

FIGURE 1

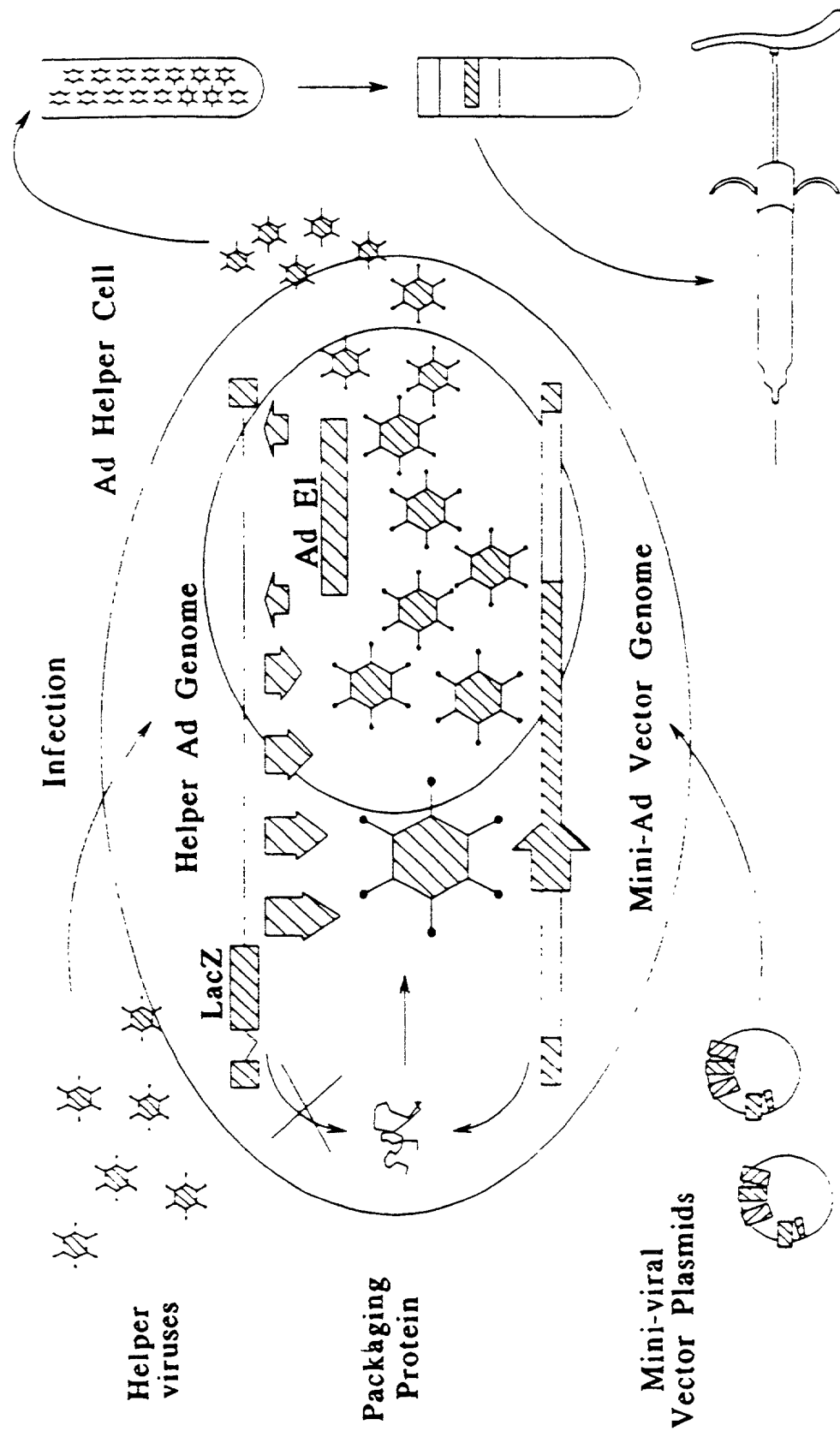


FIGURE 2

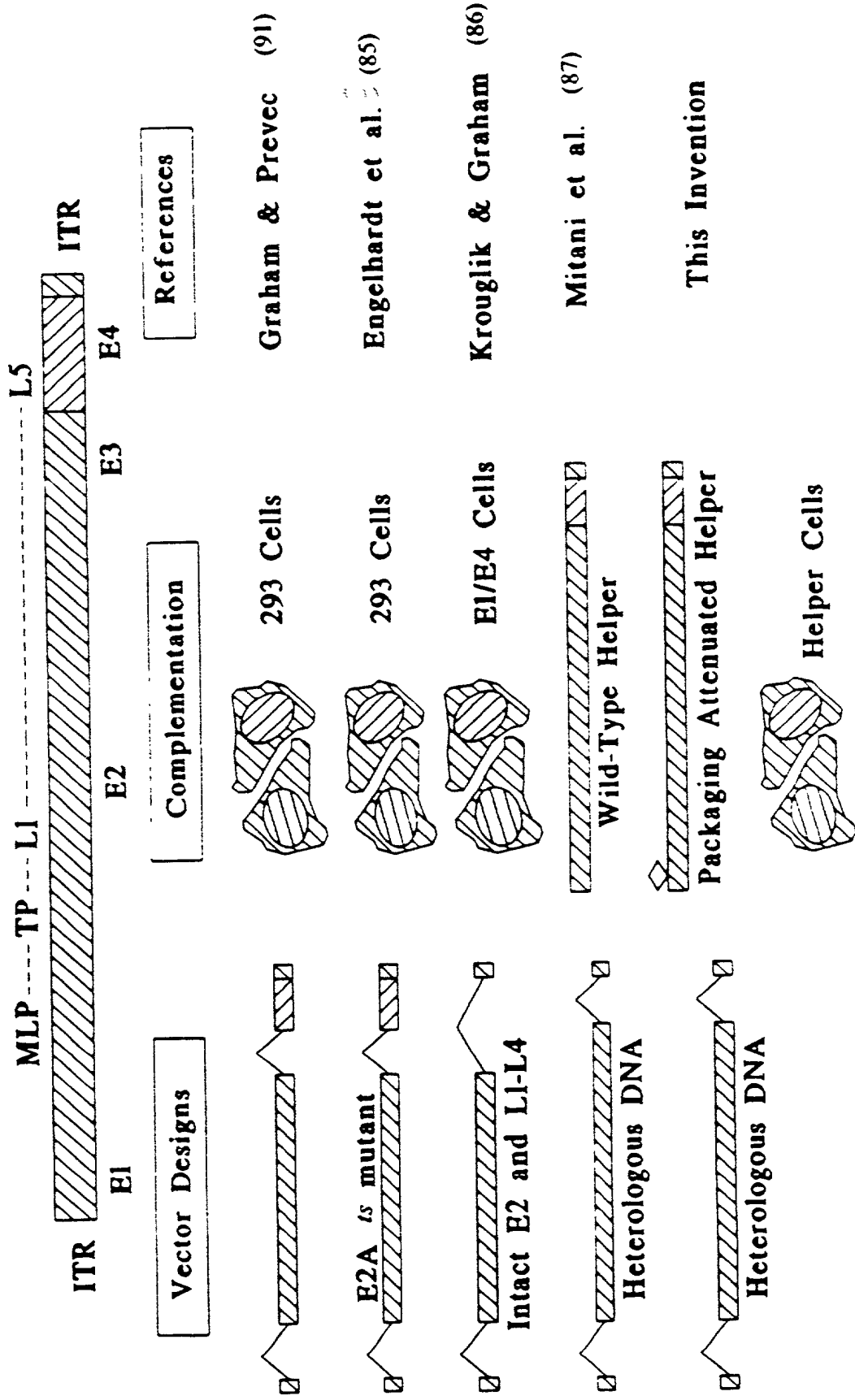


FIGURE 3

FIG. 3A

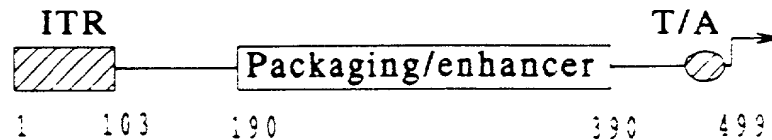


FIG. 3B

<u>E1A Core</u>		<u>E2F</u>	<u>E1A Core</u>	
GTGTACACAG	GAAGTGACAA	TTTTCGCGCG	GTTTATAGGCG	GATGTTGTAG
CACATGTGTC	CTTCACTGTT	AAAAGCGCGC	CAAAATCCGC	CTACAACATC
200	210	220	230	240
A I		A II	<u>E2F</u>	
TAAATTTGGG	CGTAACCGAG	TAAGATTTGG	CCATTTTCGC	GGGAAAACTG
ATTTAAACCC	GCATTGGCTC	ATTCTAAACC	GGTAAAAGCG	CCCTTTTGAC
250	260	270	280	290
<u>E1A Core</u>	A III	A IV		
AATAAGAGGA	AGTGAAATCT	GAATAATTTT	GTGTTACTCA	TAGCGCGTAA
TTATTCTCCT	TCACTTTAGA	CTTATTAAAA	CACAAAGAGT	ATCGCGCATT
300	310	320	330	340
A V	A VI	A VII		
TATTTGTCTA	GGGCGCGGG	GACTTTGACC	GTTTACGTGG	AGACTCGAAA
ATAAACAGAT	CCCGGCGCCC	CTGAAACTGG	CAAATGCACC	TCTGAGCGGG
350	360	370	380	390

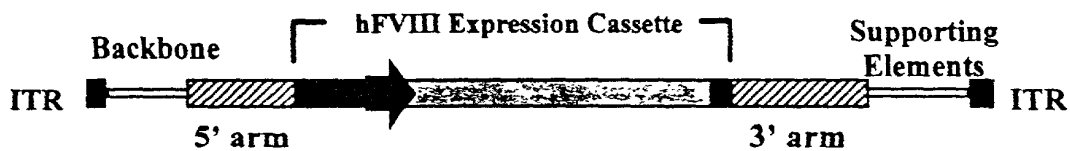
FIG. 3C

A repeat I	5'-GTAAATTTG-3'
A repeat II	GTAAAGATTTG
A repeat III	GTCAAAATCTG
A repeat IV	ATAATTTTG
A repeat V	GTAATAATTTG
A repeat VI	GGGACTTTTG
A repeat VII	GACCGTTTA

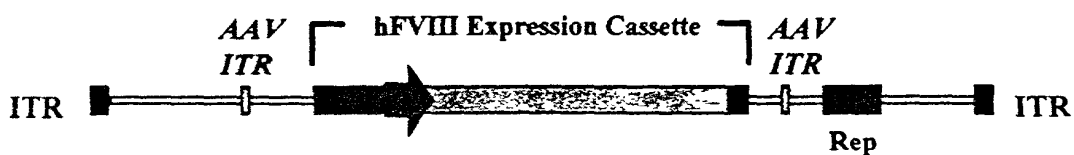
CONSENSUS: 5'-GT-N_{1,2} TTTG-3'

FIGURE 3D

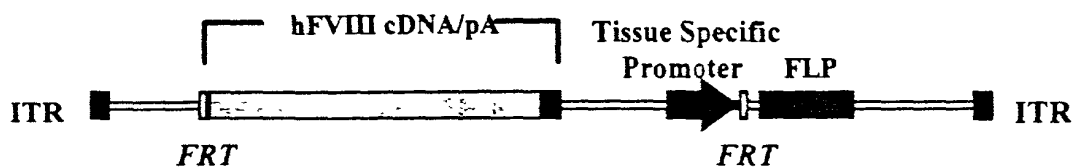
Product Design for MiniAdFVIII



Version 1: Genomic Sequence Device



Version 2: AAV/ITR Integration Device



Version 3: Episomal Device

FIGURE 4

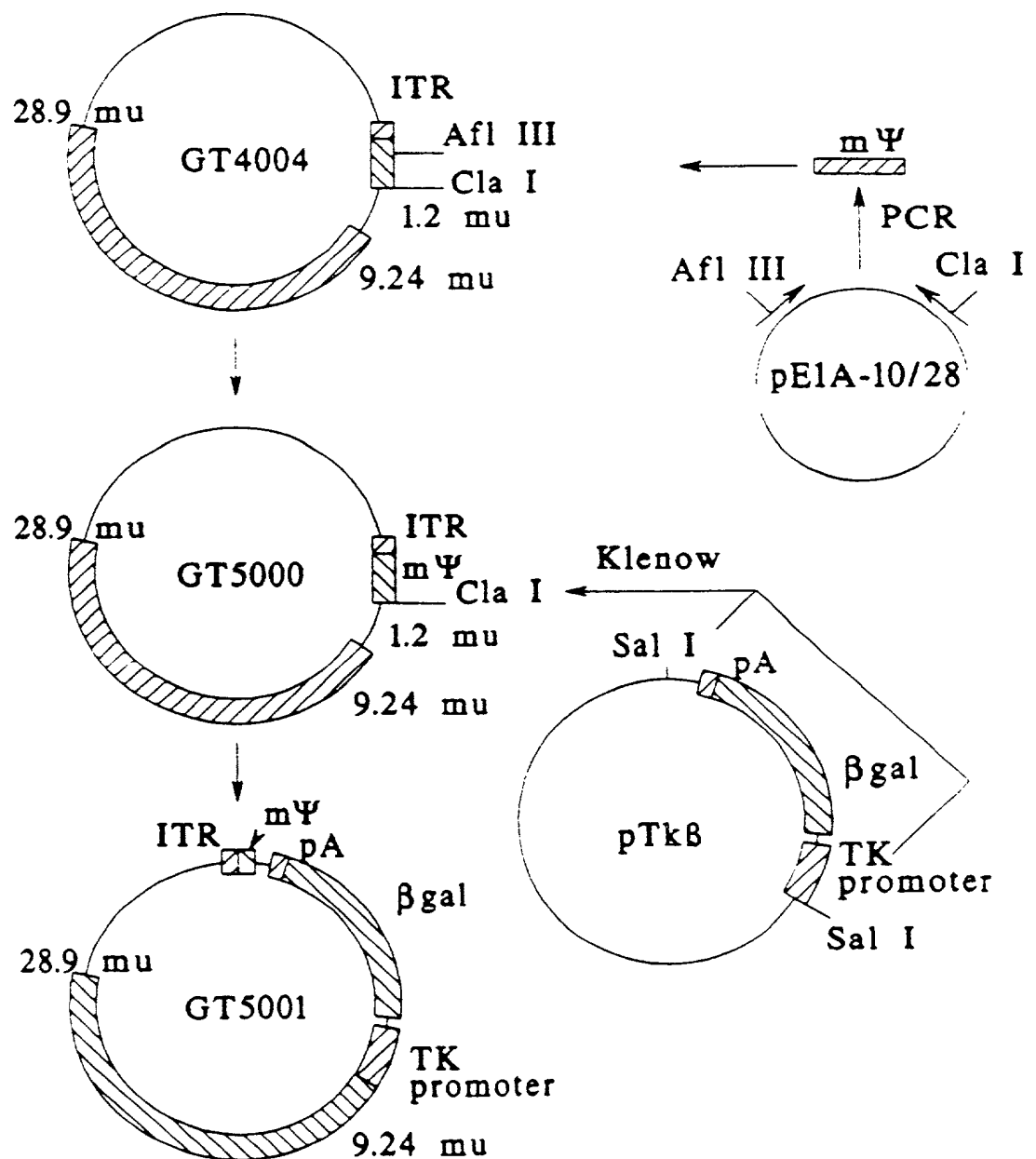


FIGURE 5A

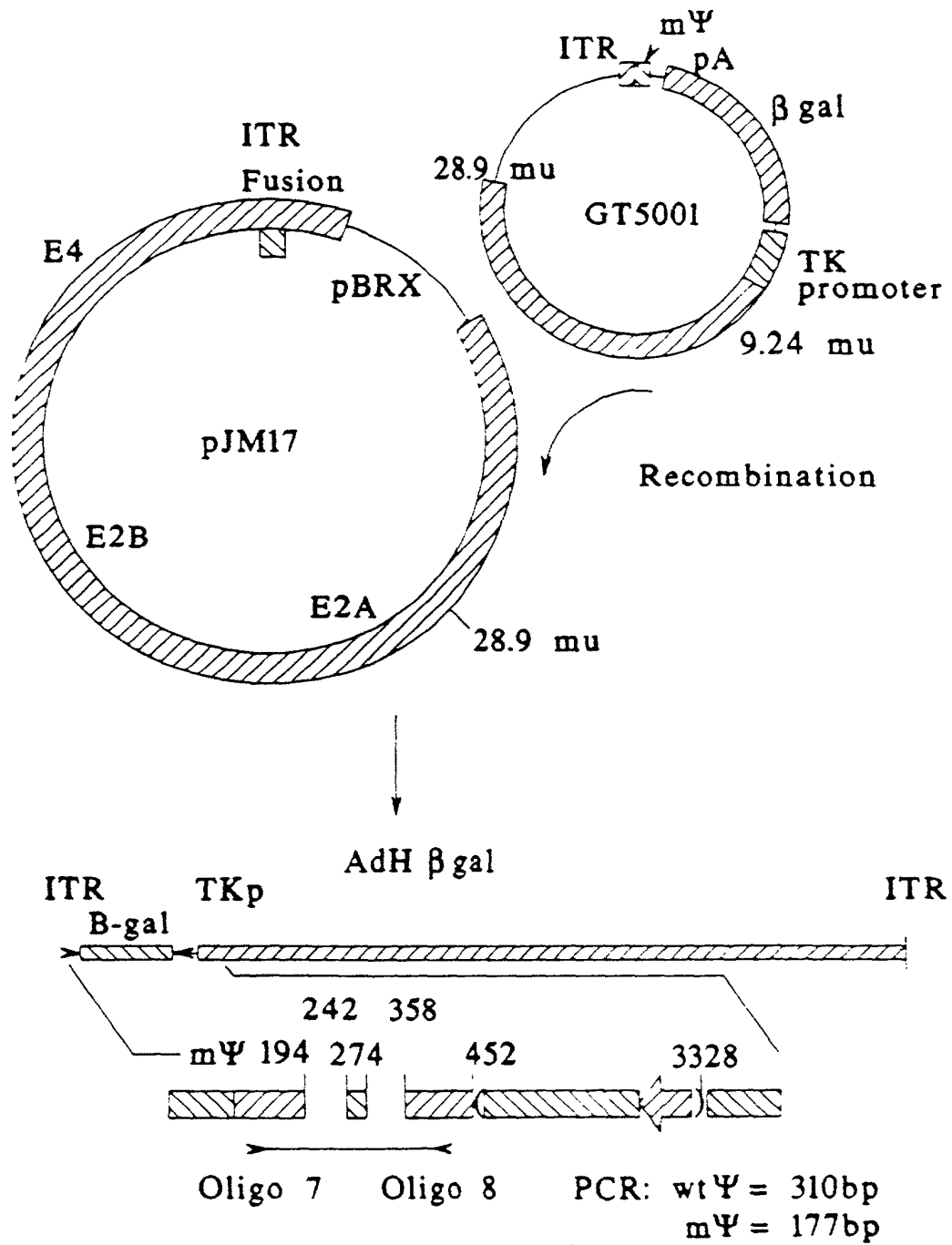


FIGURE 5B

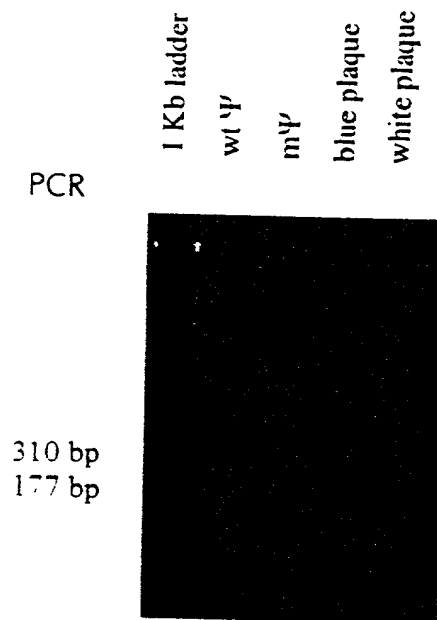


FIGURE 6

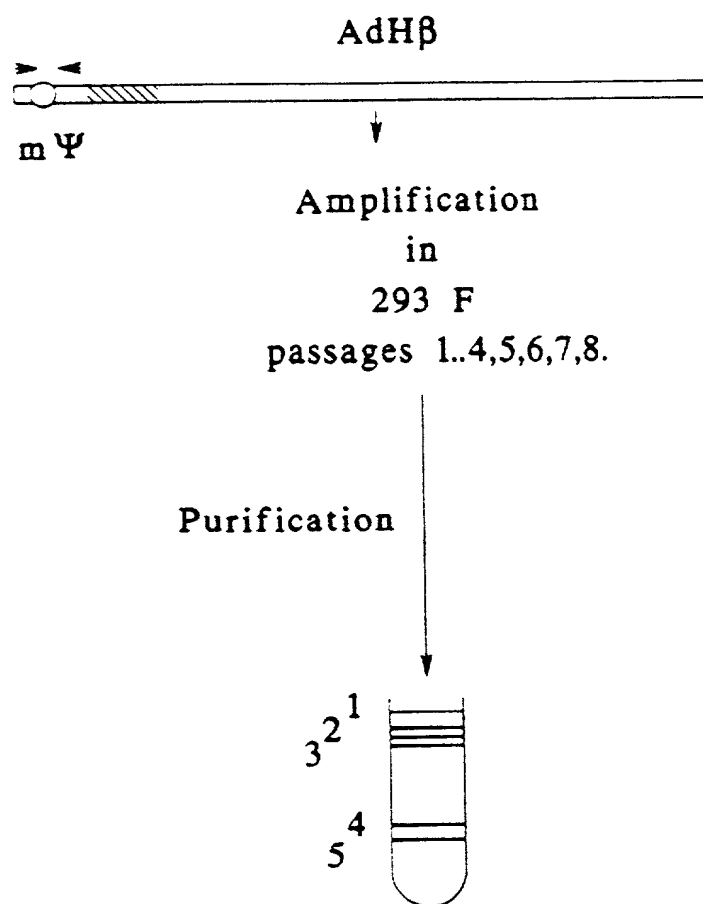


FIGURE 7

FIG. 7A

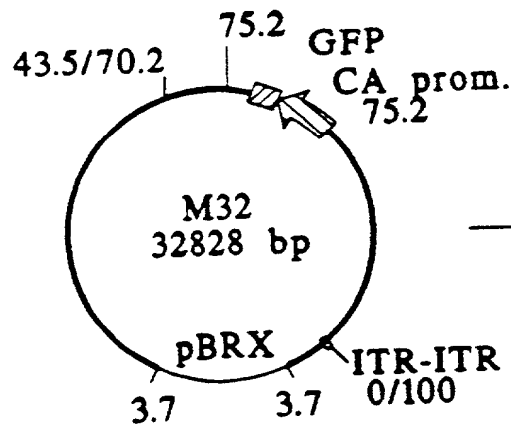


FIG. 7B

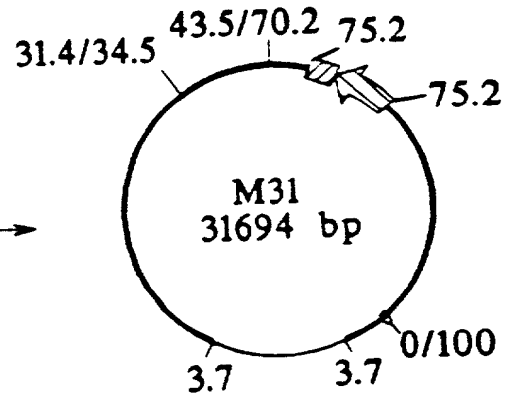


FIG. 7C

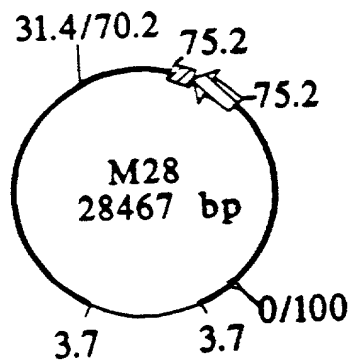


FIG. 7D

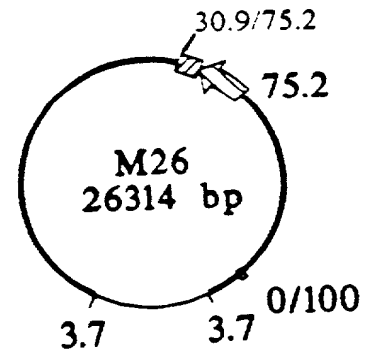


FIG. 7E

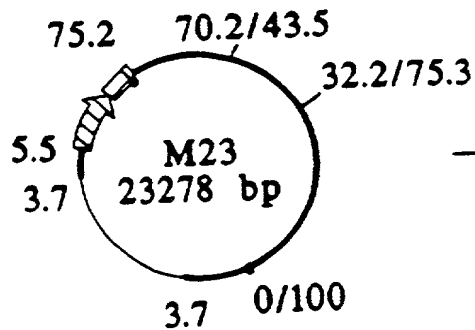


FIG. 7F

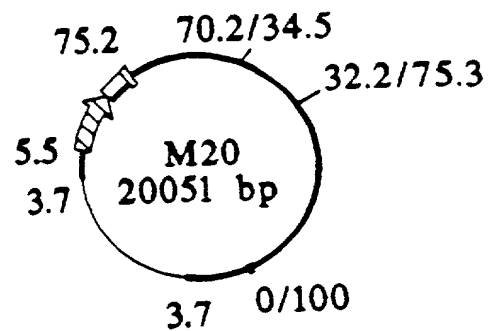


FIG. 7G

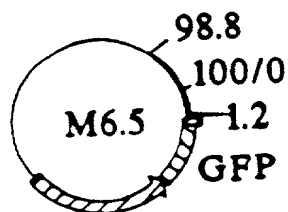


FIG. 7H

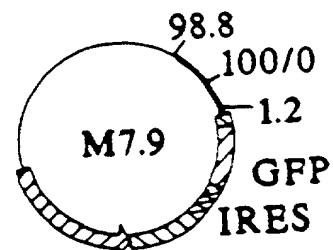


FIGURE 8

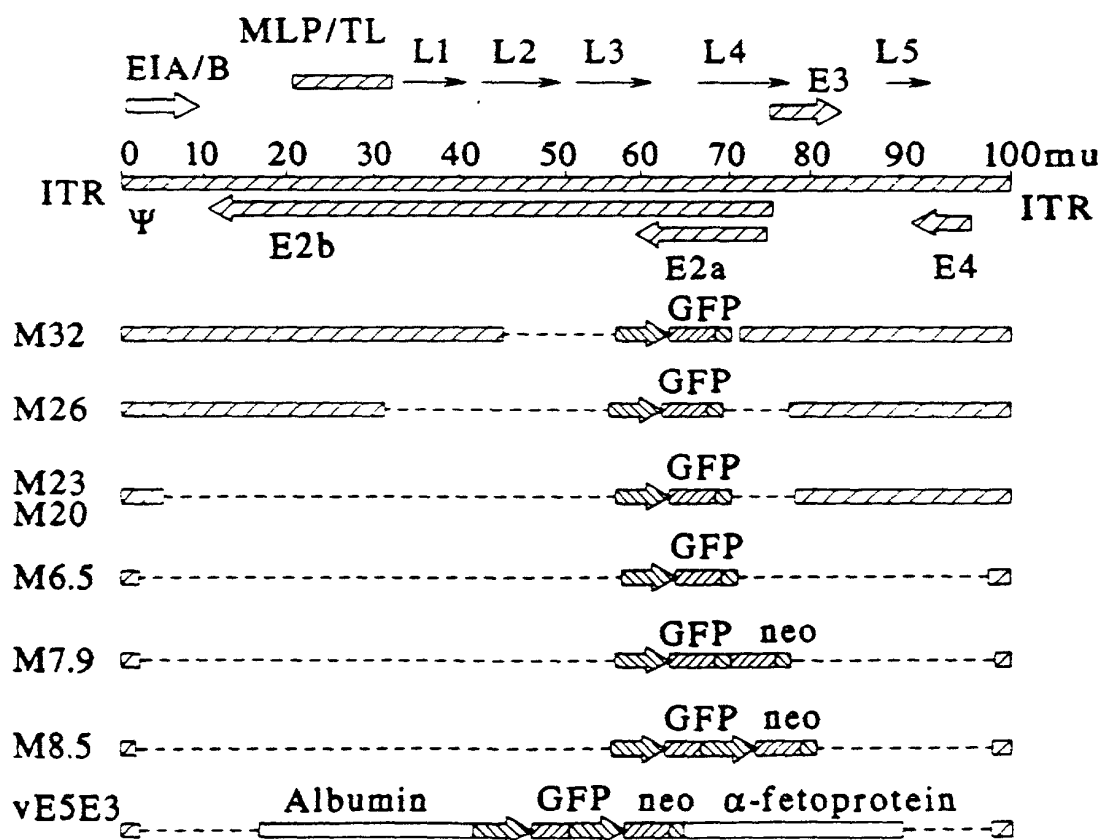


FIGURE 9

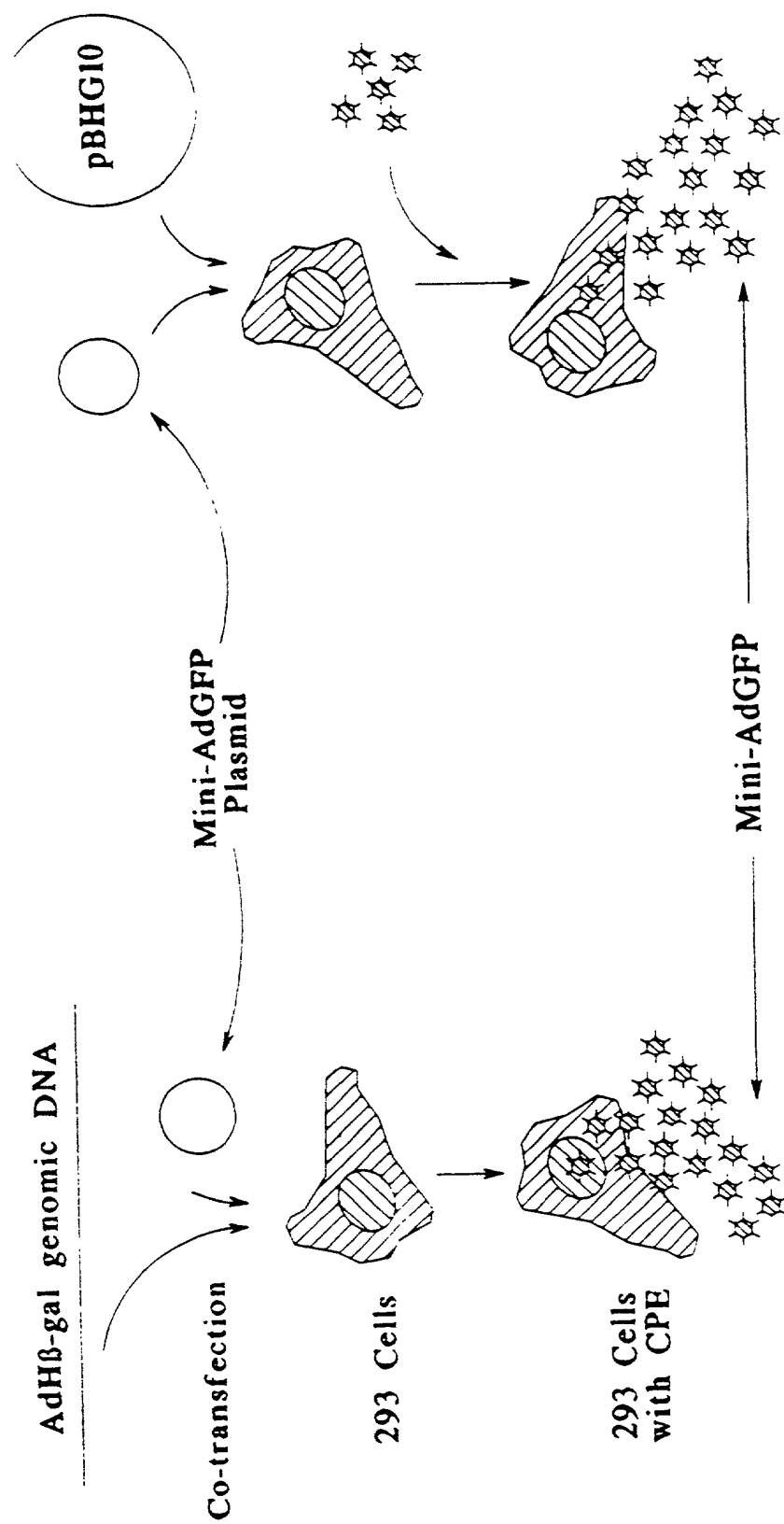


FIGURE 10

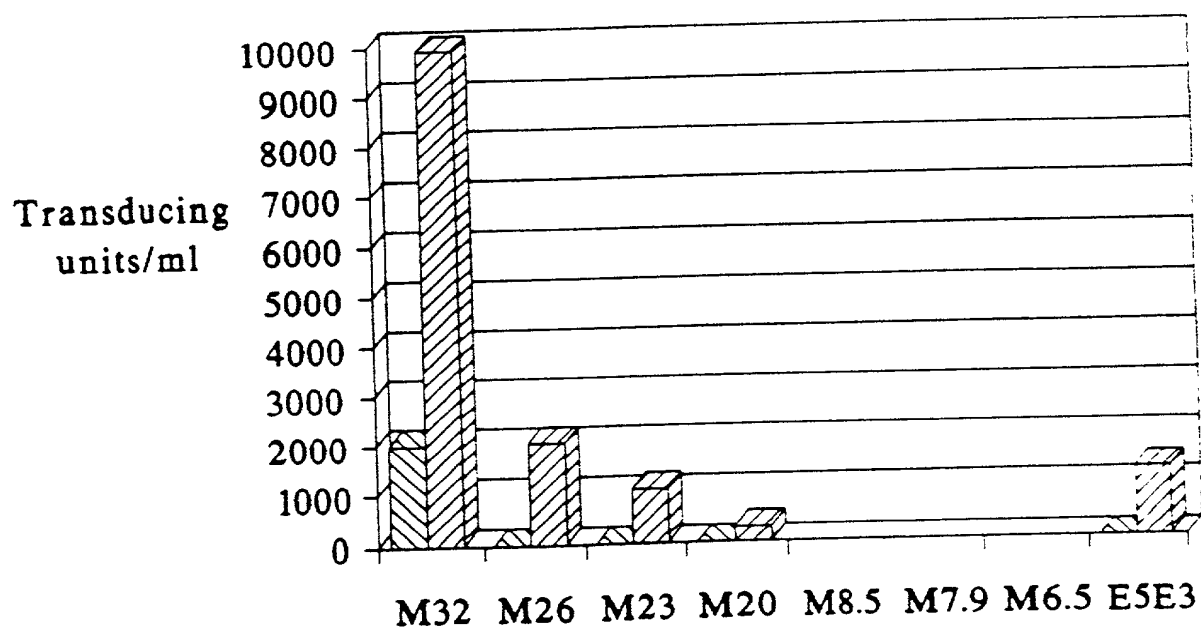
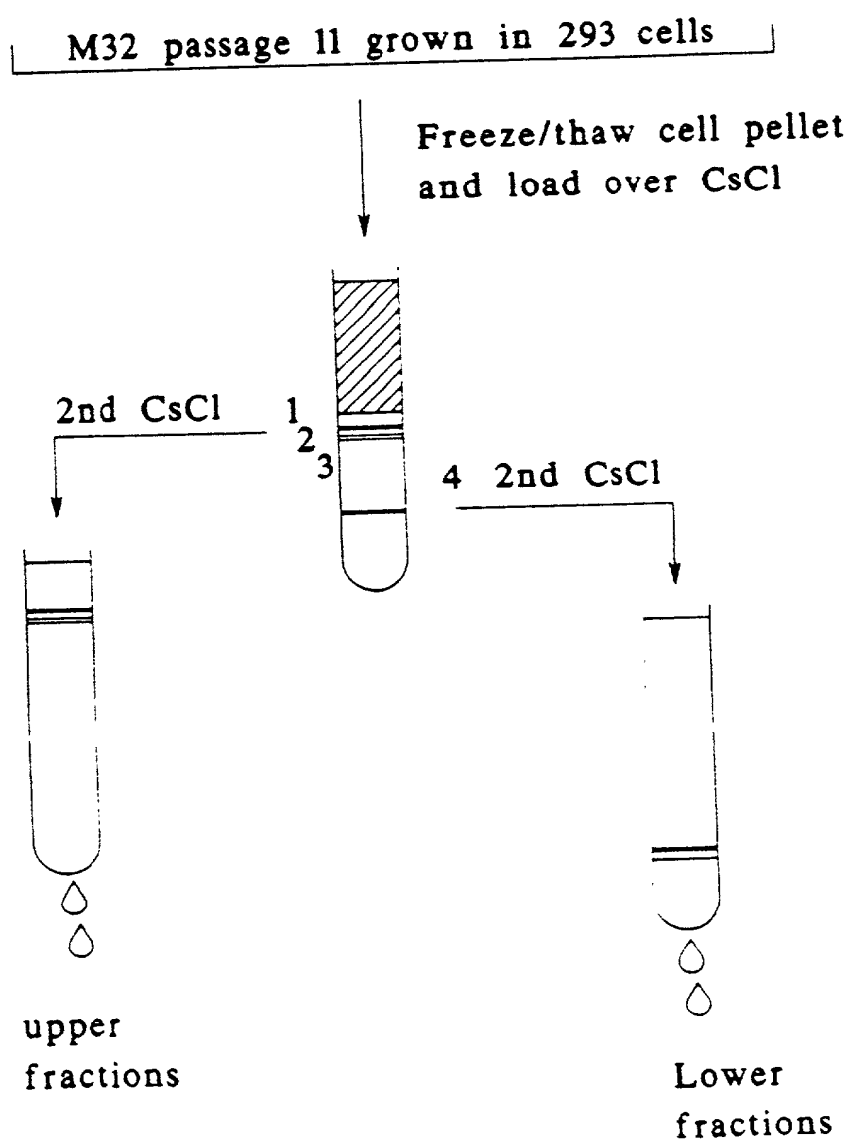


FIGURE 11



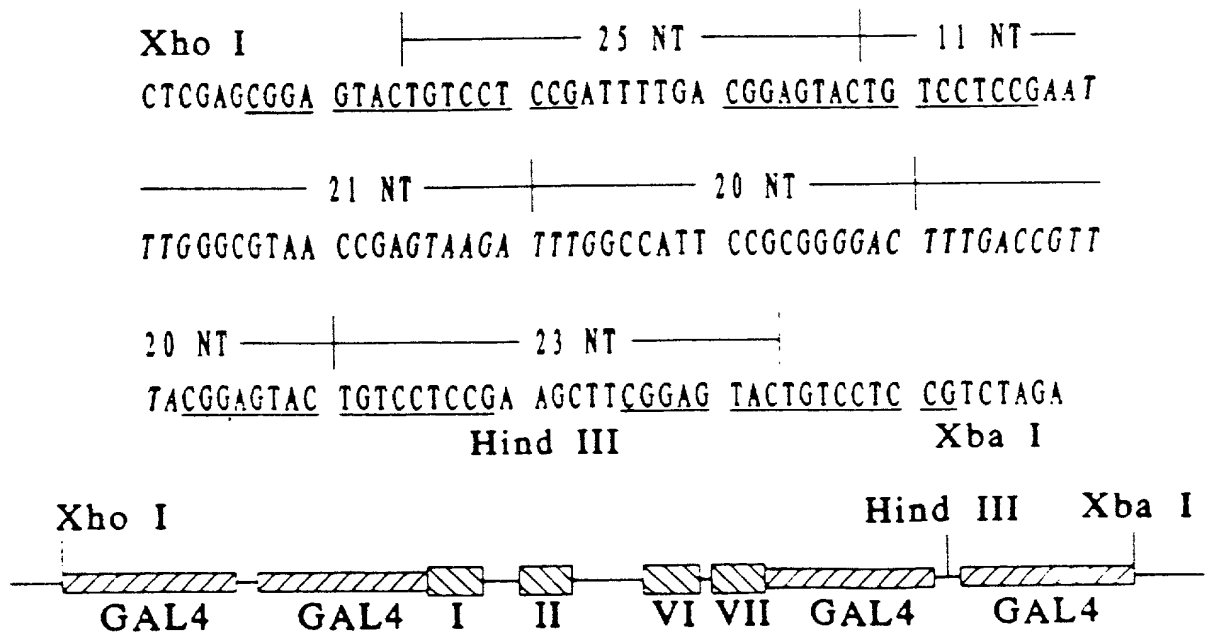
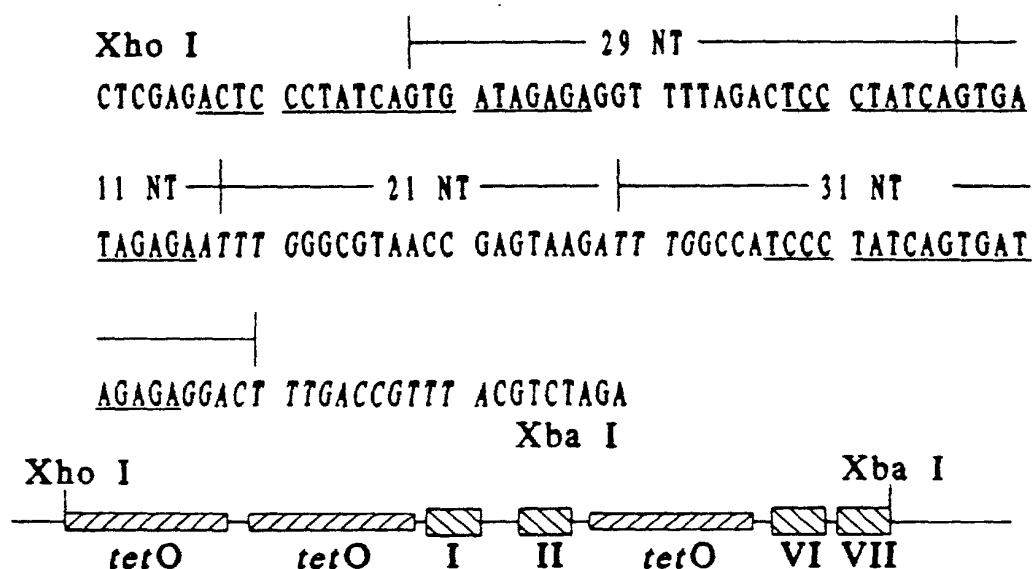
[illegible]

FIGURE 13

Ad5 Pac Modification with *tetO* Binding Sites

Design #1



Design #2

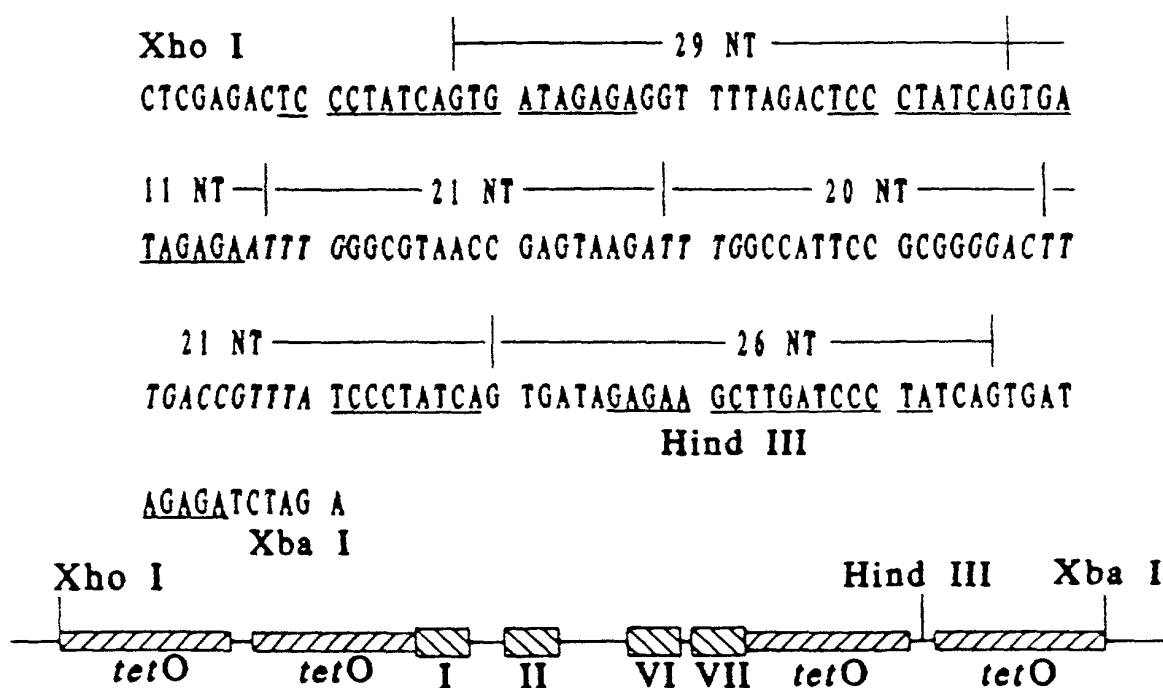


FIGURE 14

Positions and Sequences of Synthetic Oligos for Ad Pac-GAL4 Modification
(Design #1 & #2)

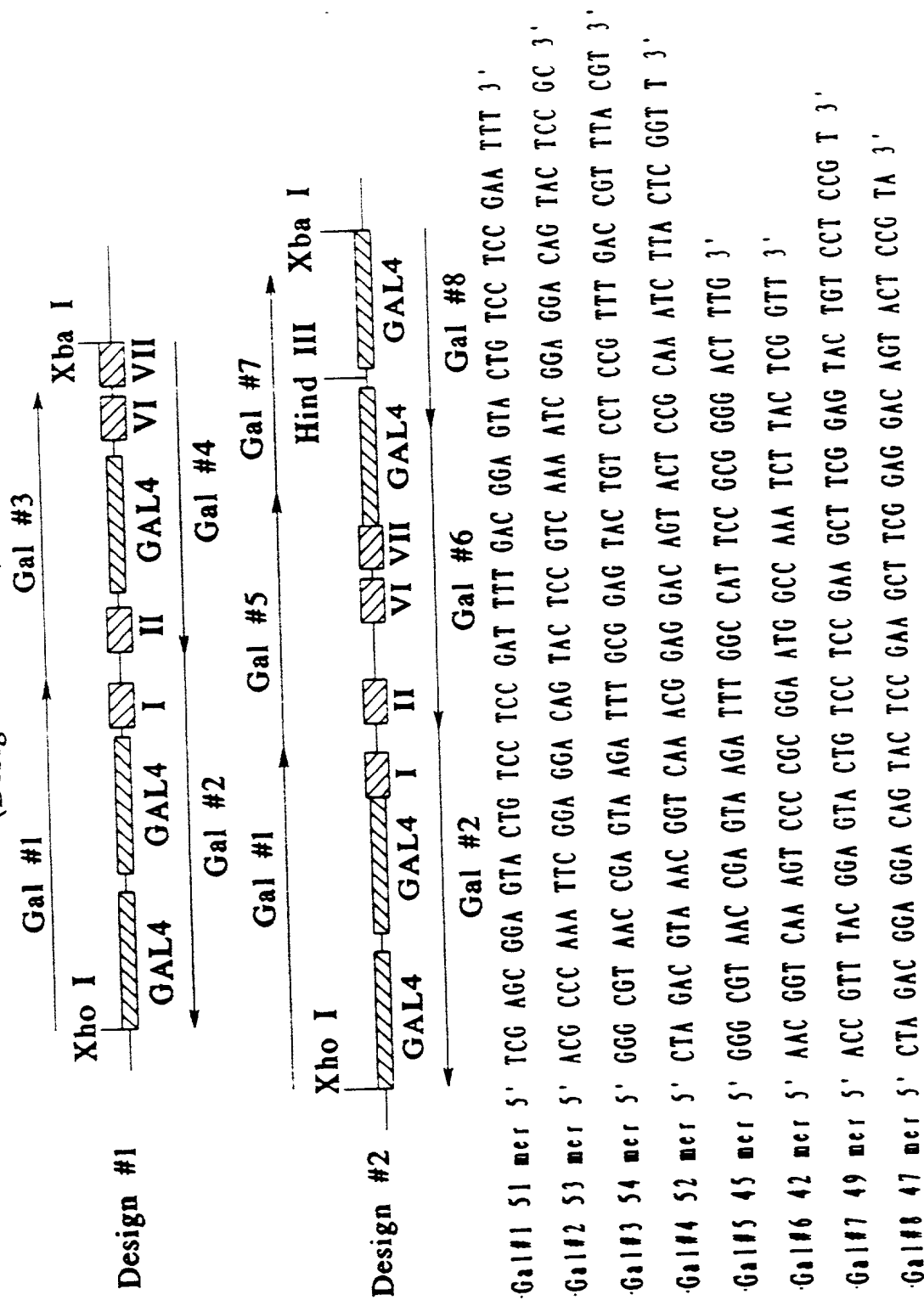


FIGURE 15

Positions and Sequences of Synthetic Oligos for Ad Pac- *tetO* Modification (Design #1 & #2)

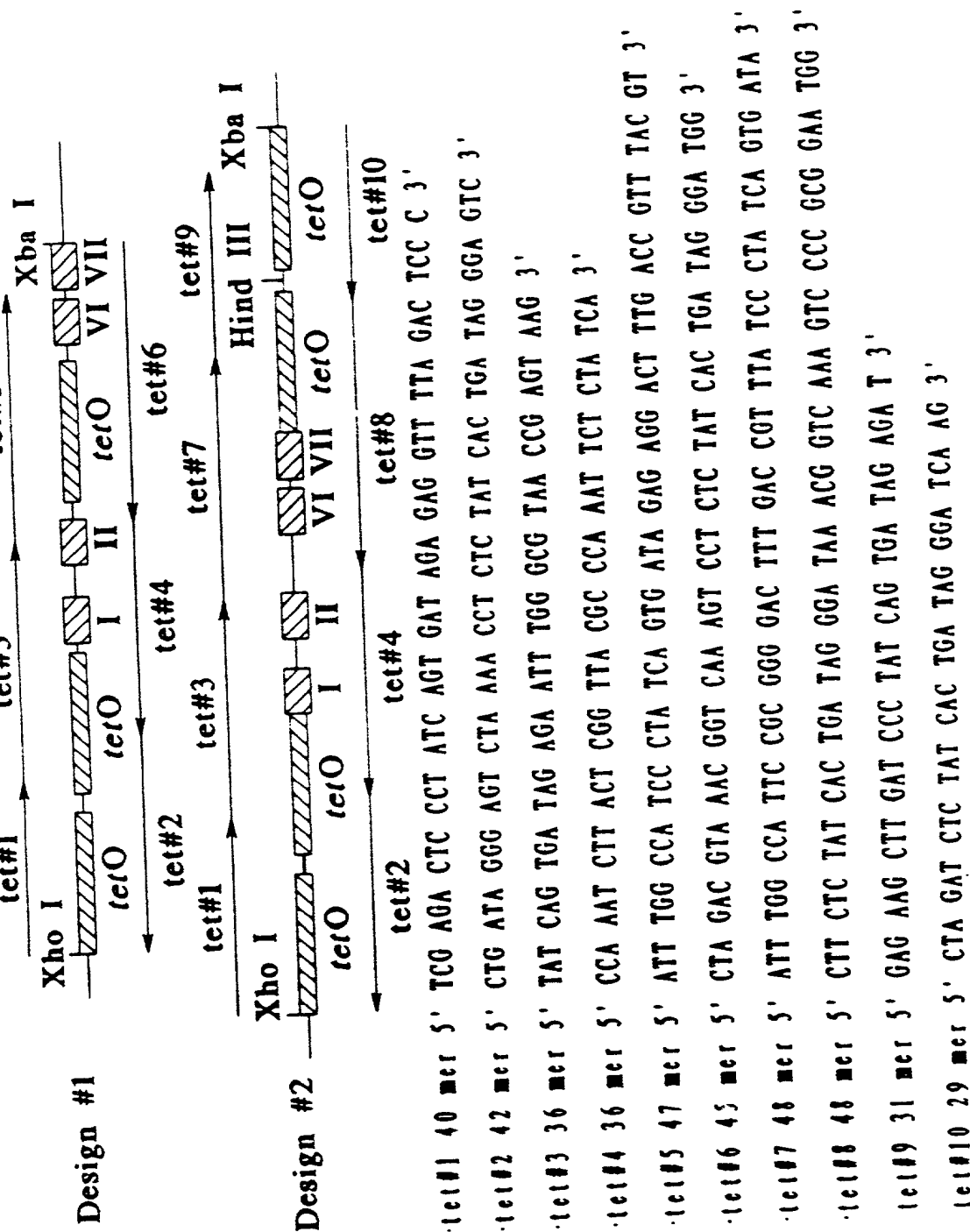


FIGURE 16

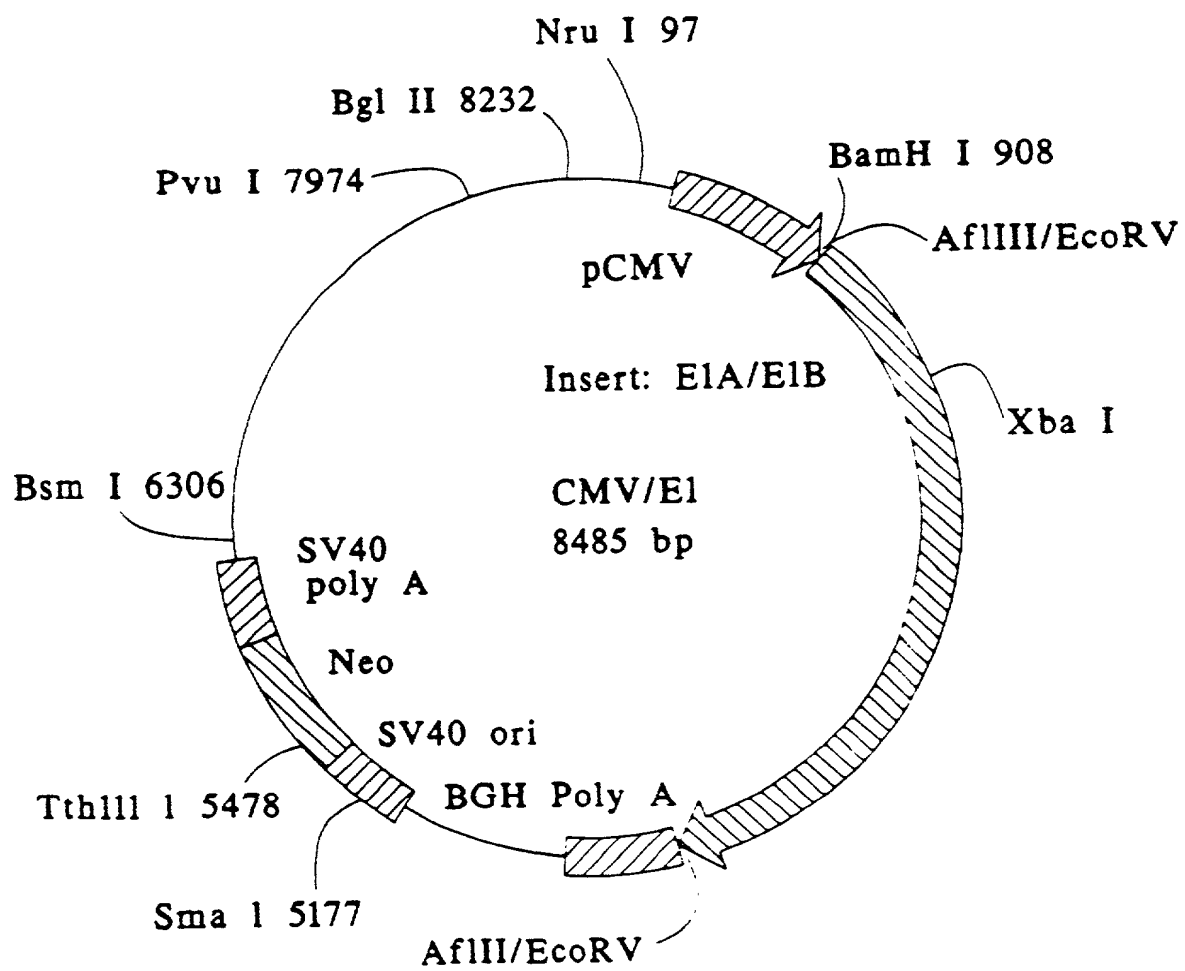
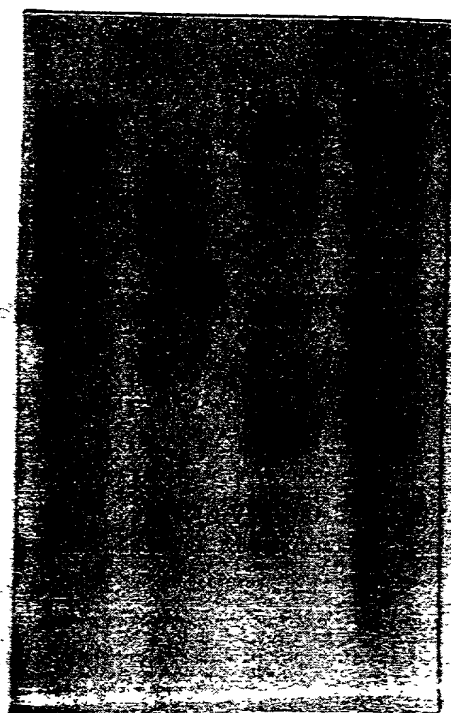


FIGURE 17

1 2 3 4



- 3.0 kb

FIGURE 18

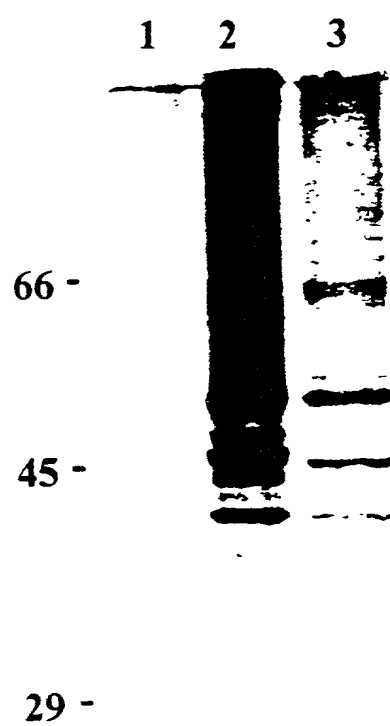


FIGURE 19

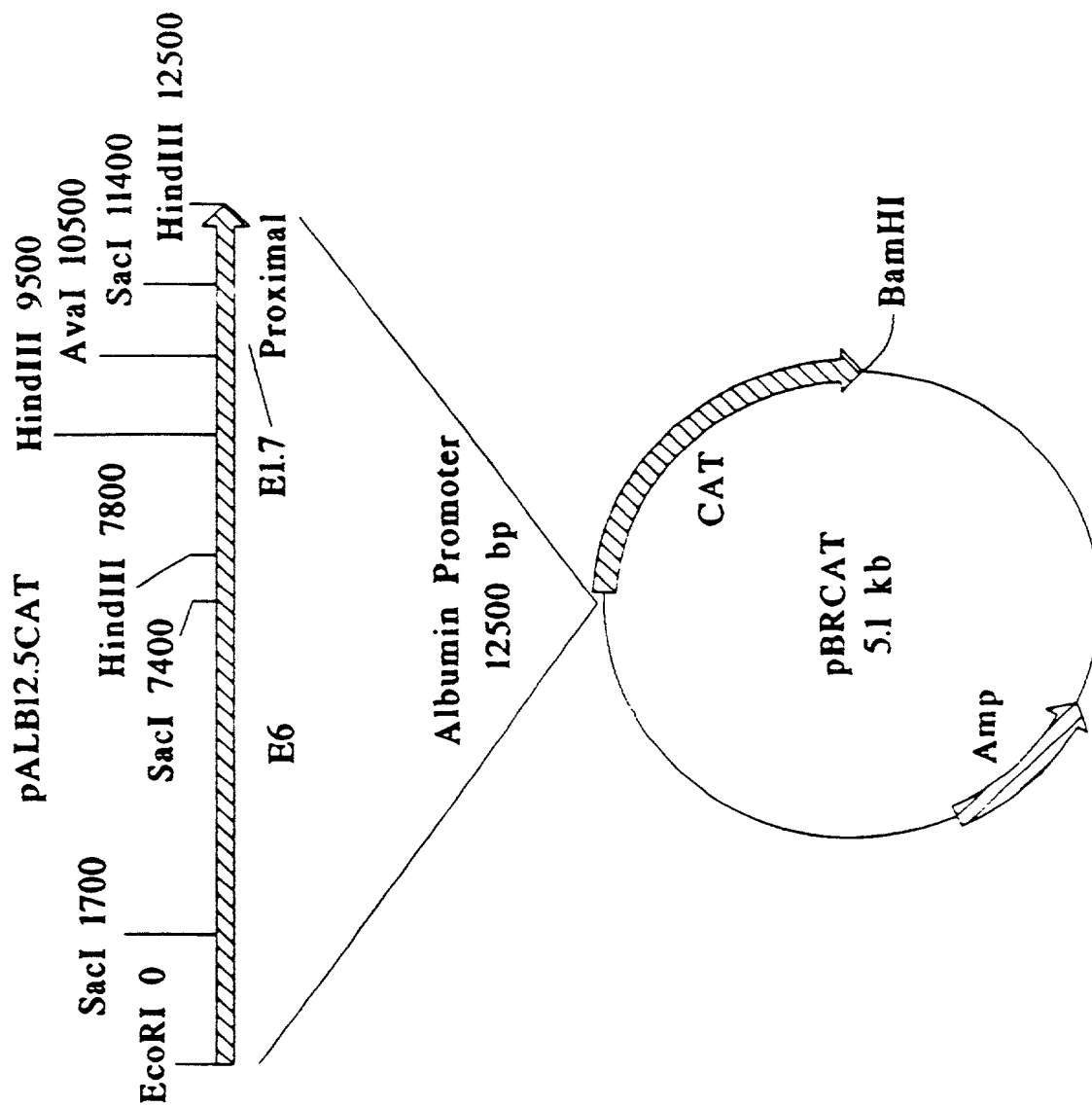


FIGURE 20

Albumin 12.5 kb Promoter Fragments

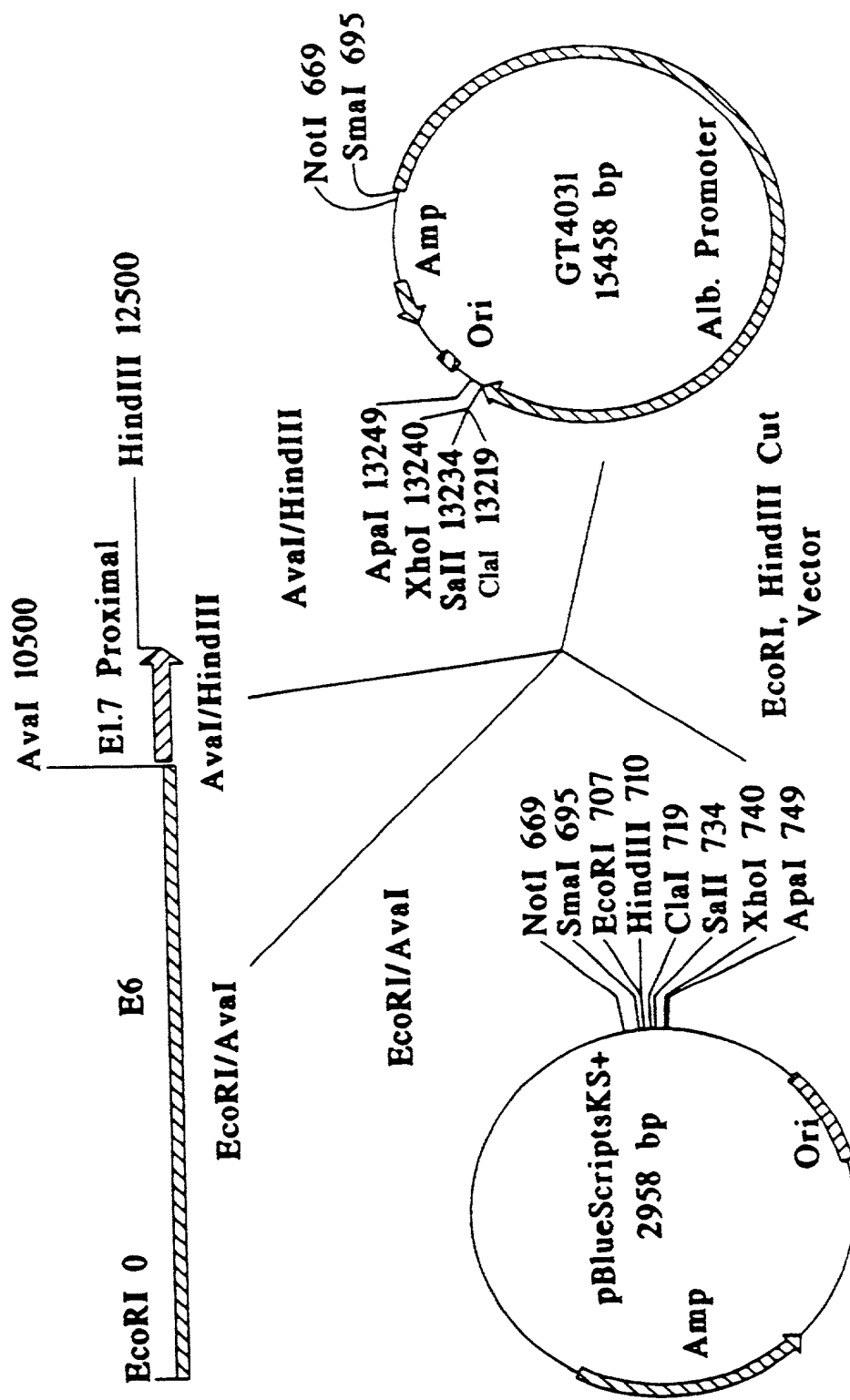


FIGURE 21

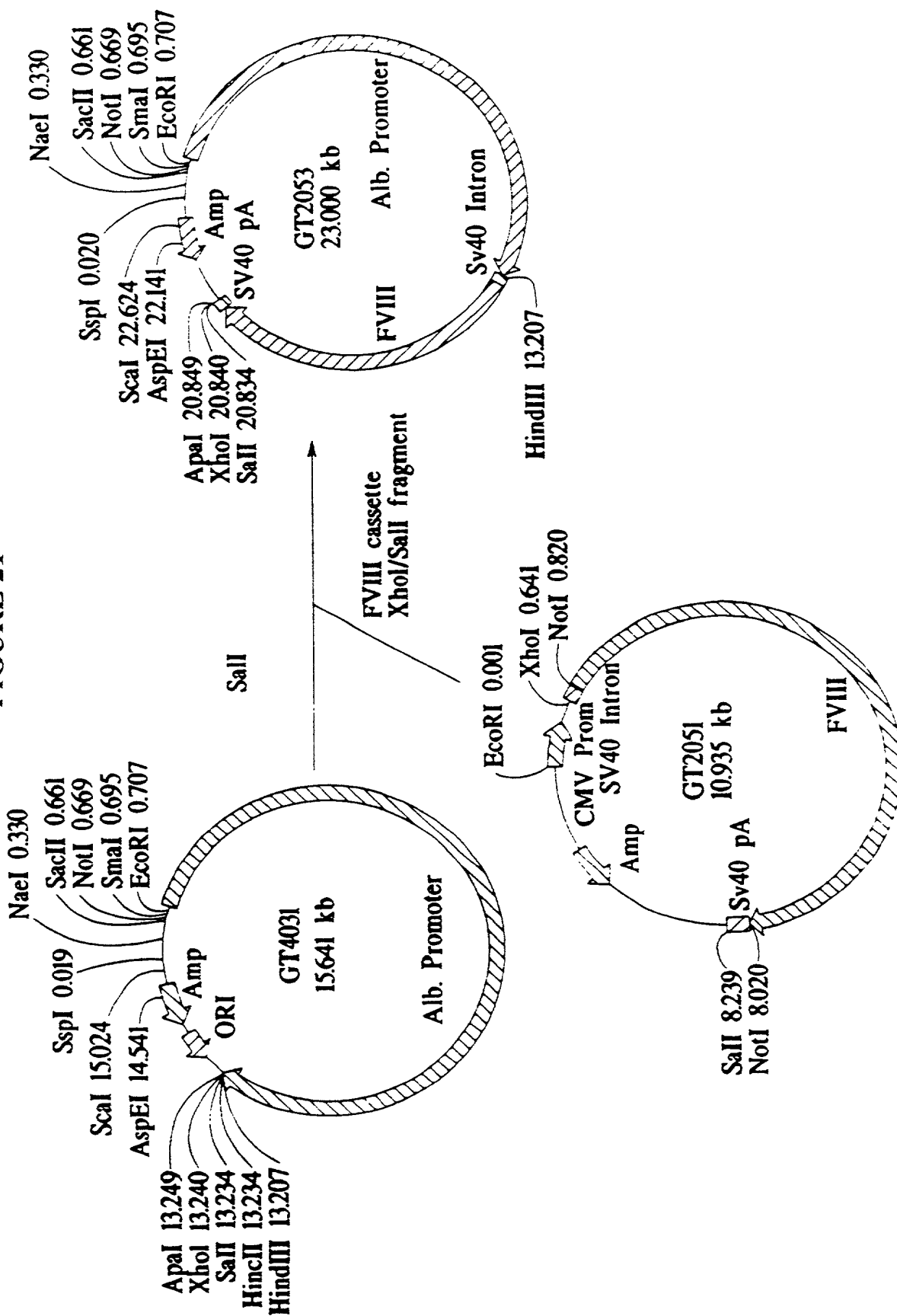


FIGURE 22

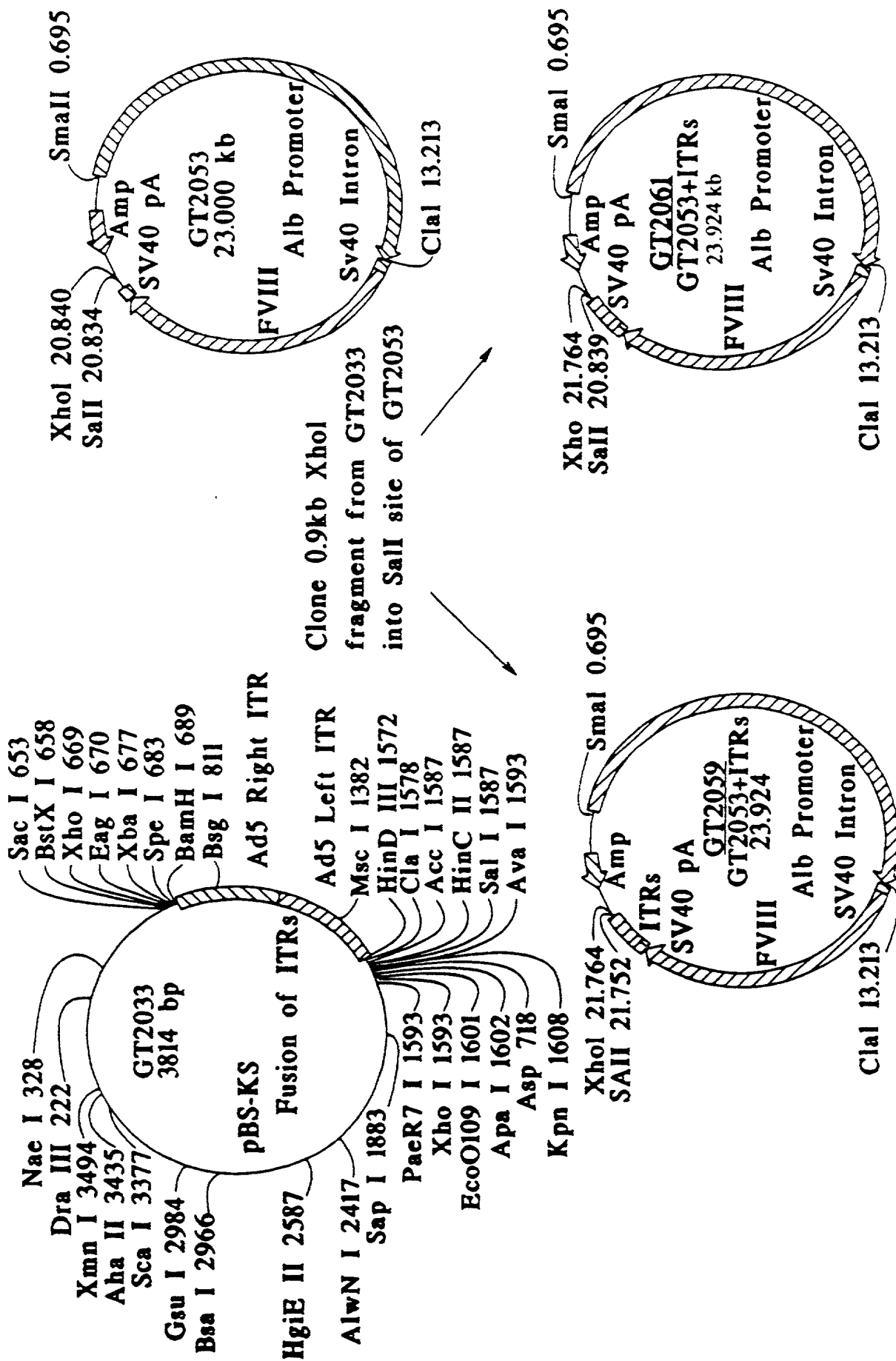
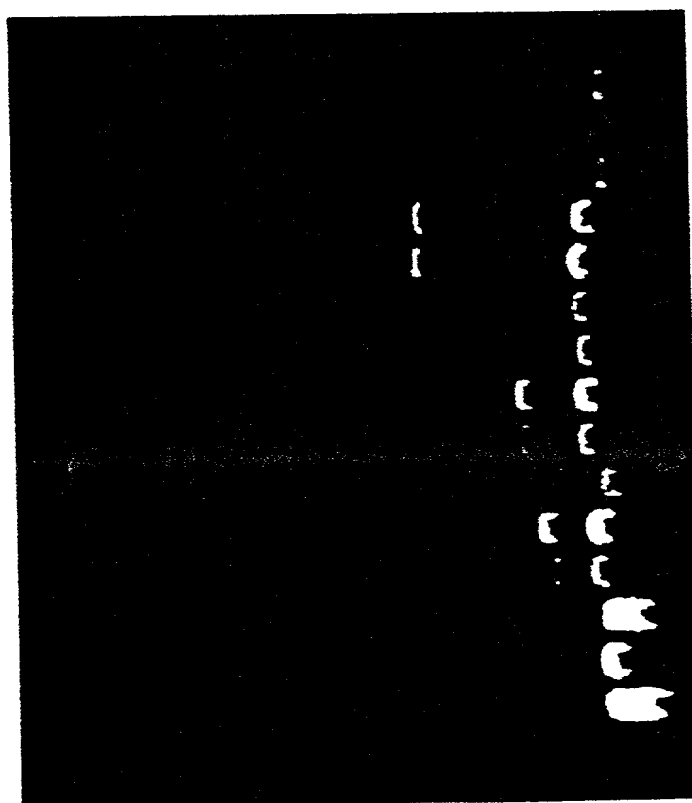


FIGURE 23



1 Kb DNA Marker

GT2053

GT2061

GT2059

GT2053 BamHI

GT2061 BamHI

GT2059 BamHI

GT2053 XbaI

GT2061 XbaI

GT2059 XbaI

GT2053 ClaI

GT2061 ClaI

GT2059 ClaI

GT2053 XhoI + Sall

GT2061 XhoI + Sall

GT2059 XhoI + Sall

1Kb DNA marker

FIGURE 24

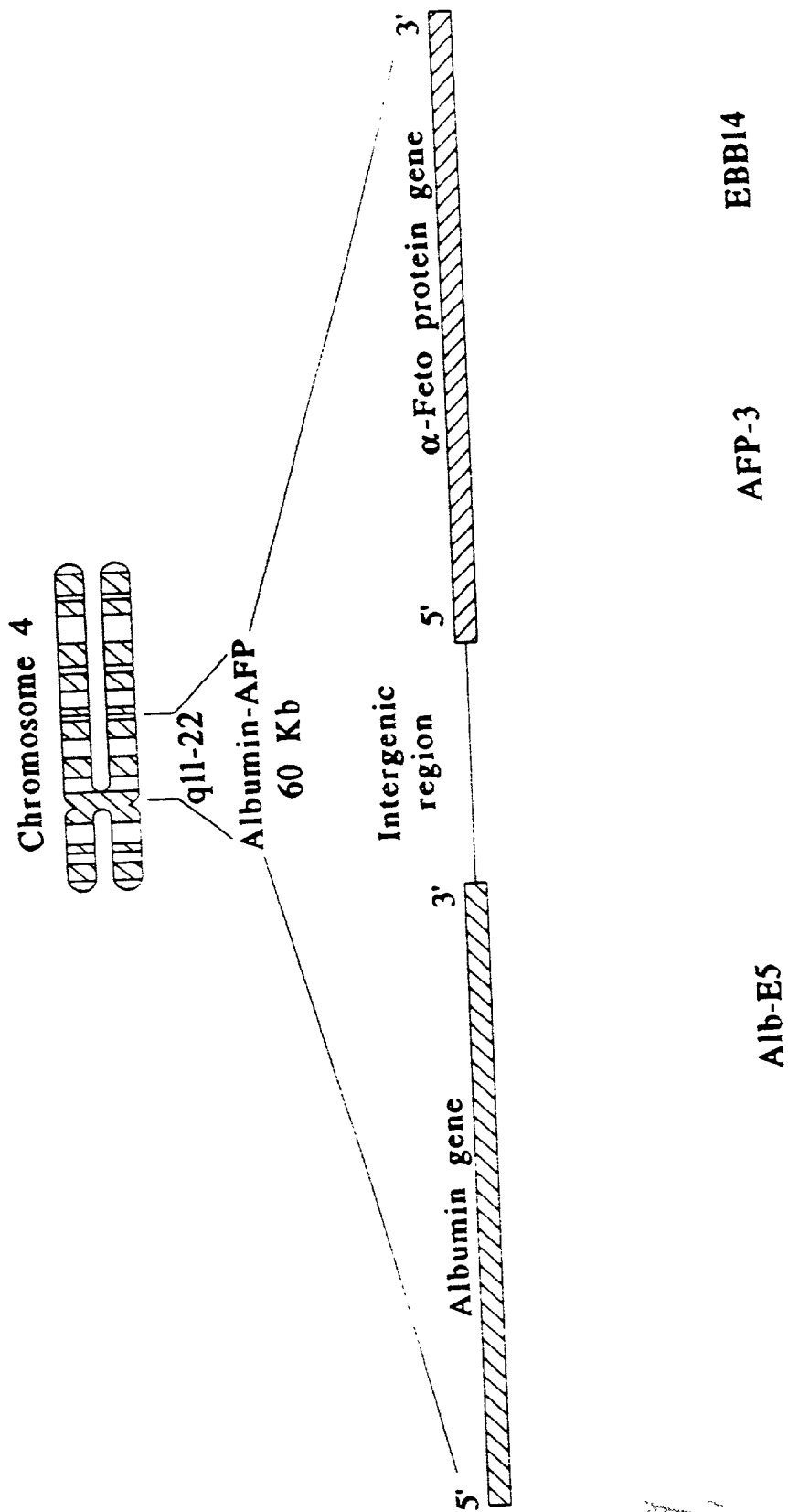
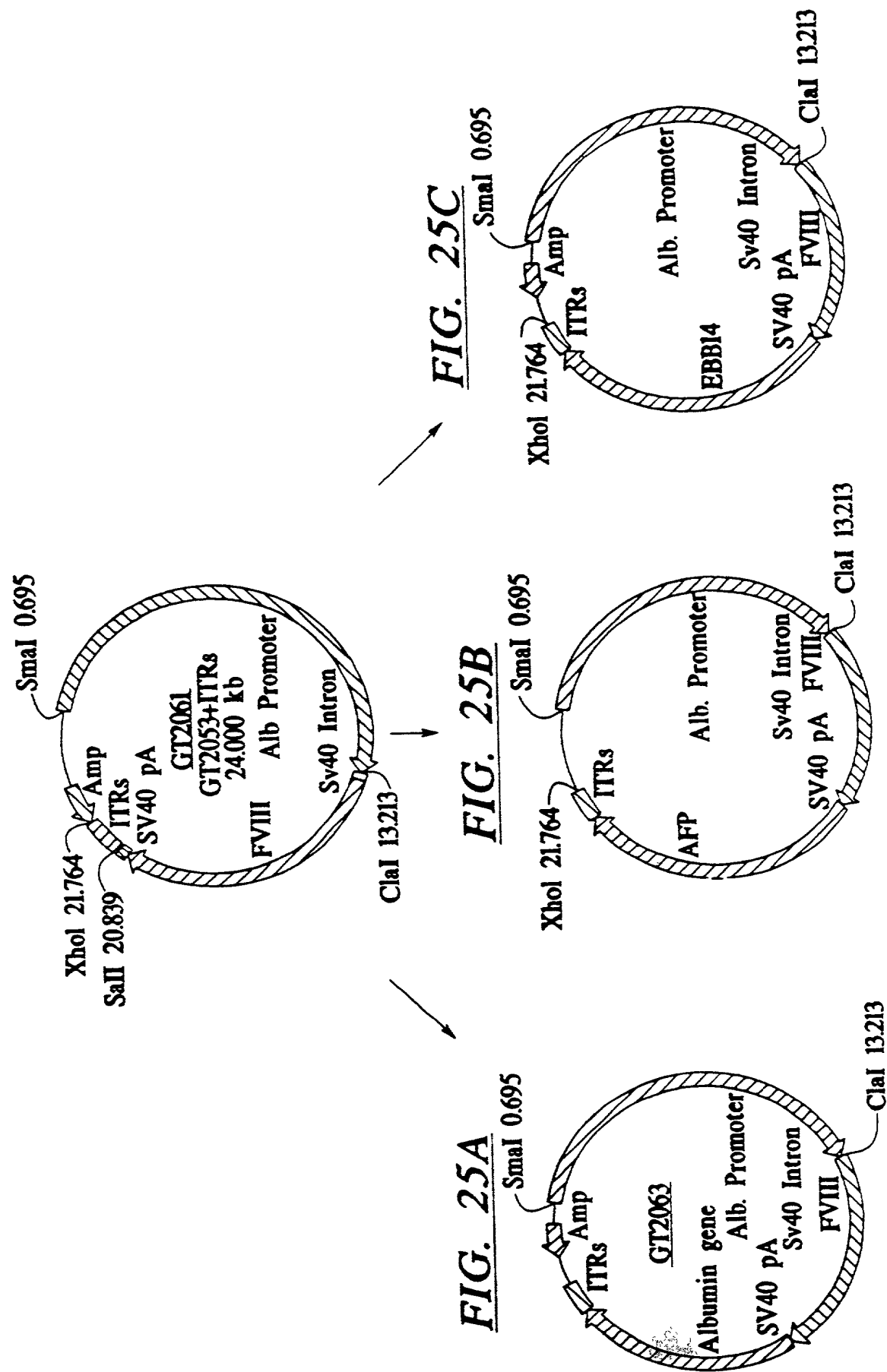


FIGURE 25



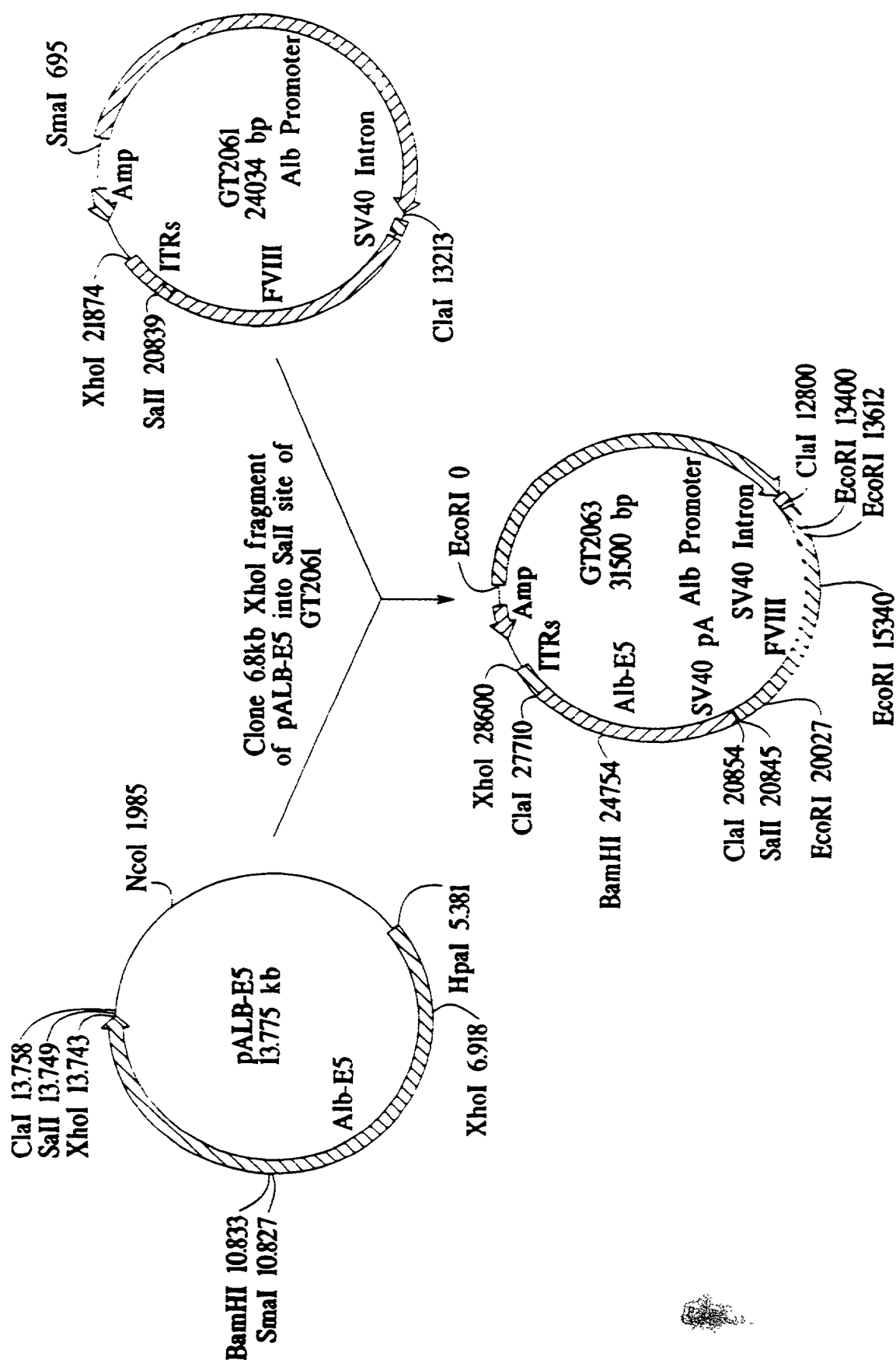


FIGURE 27

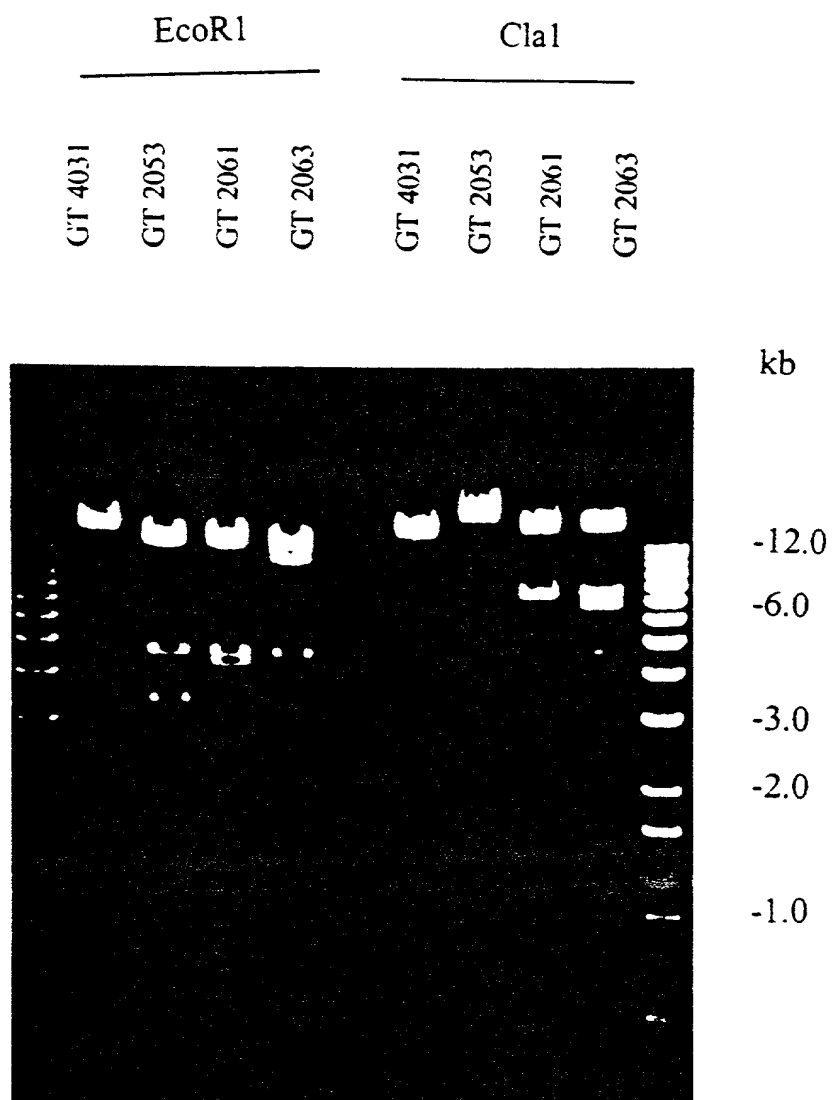


FIGURE 28

FIG. 28A

FIG. 28B

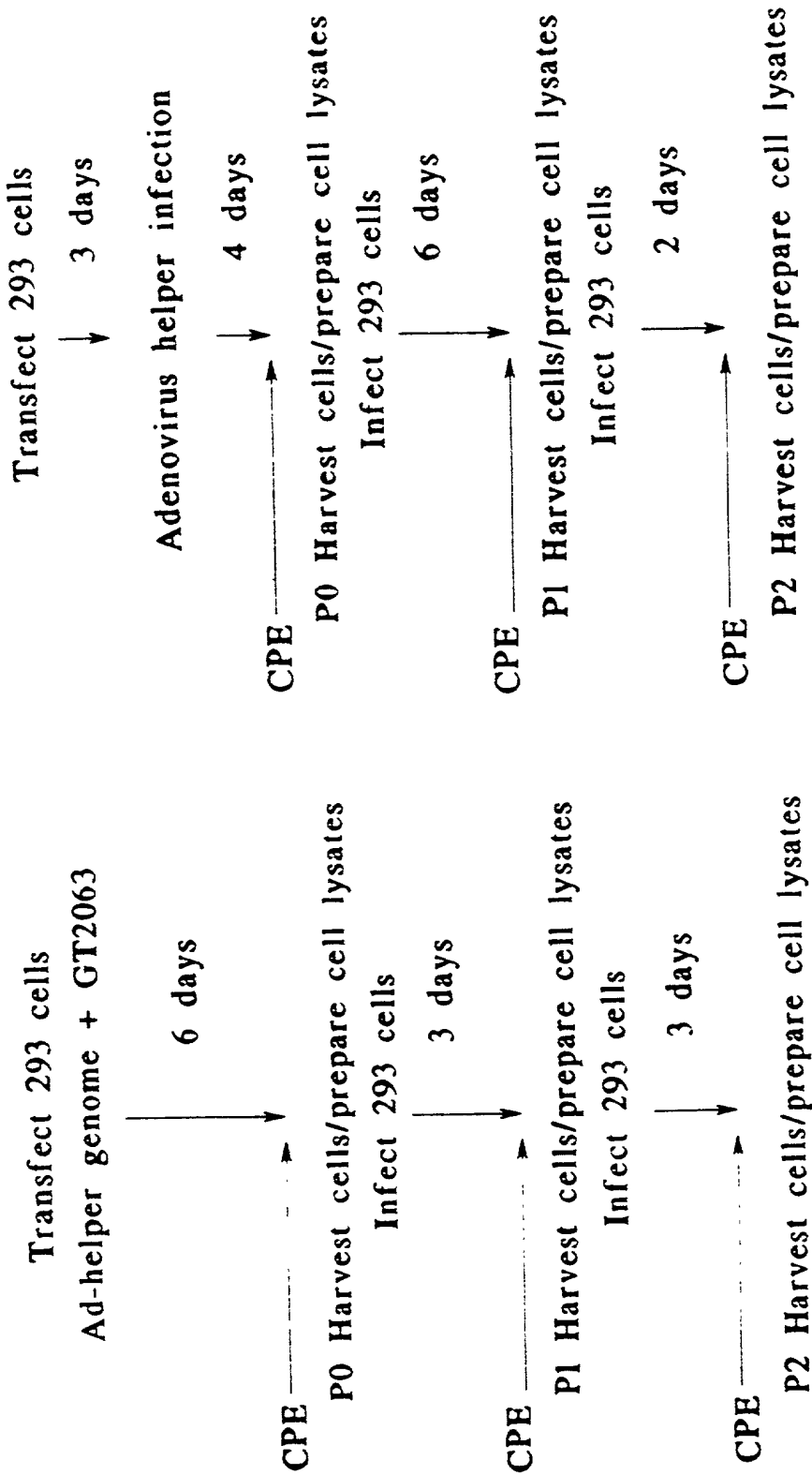


FIGURE 29

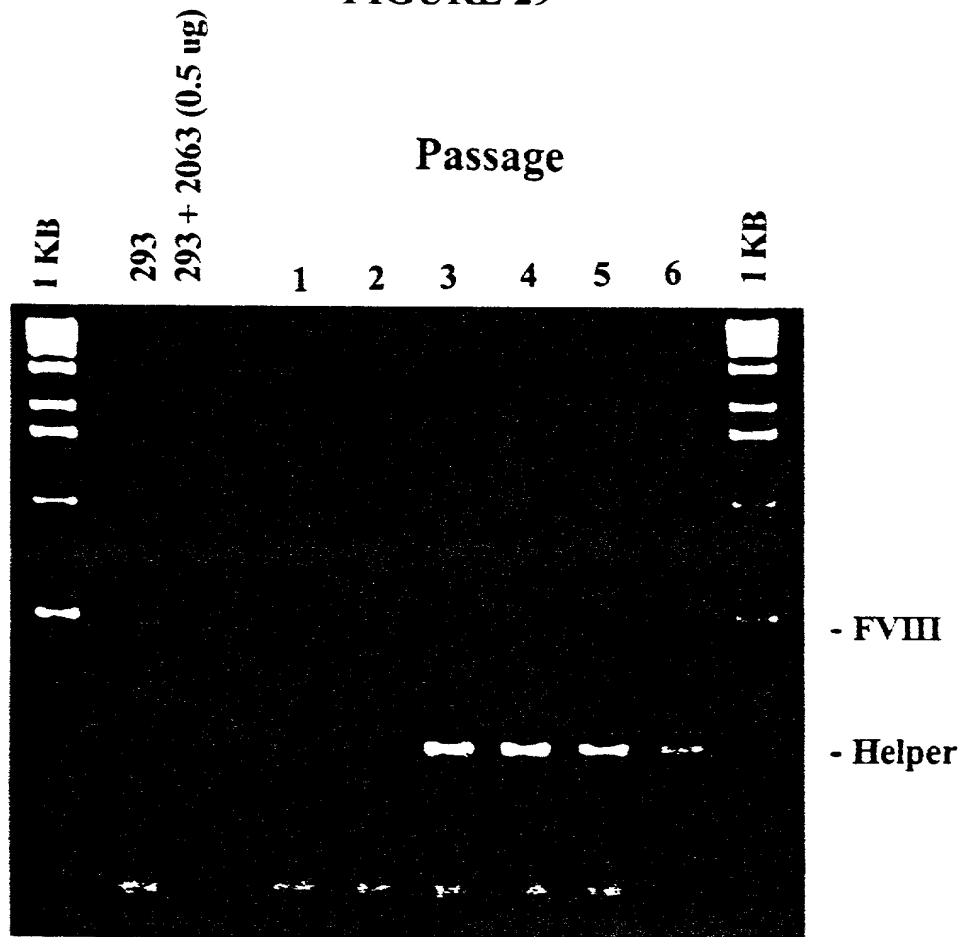


FIGURE 30

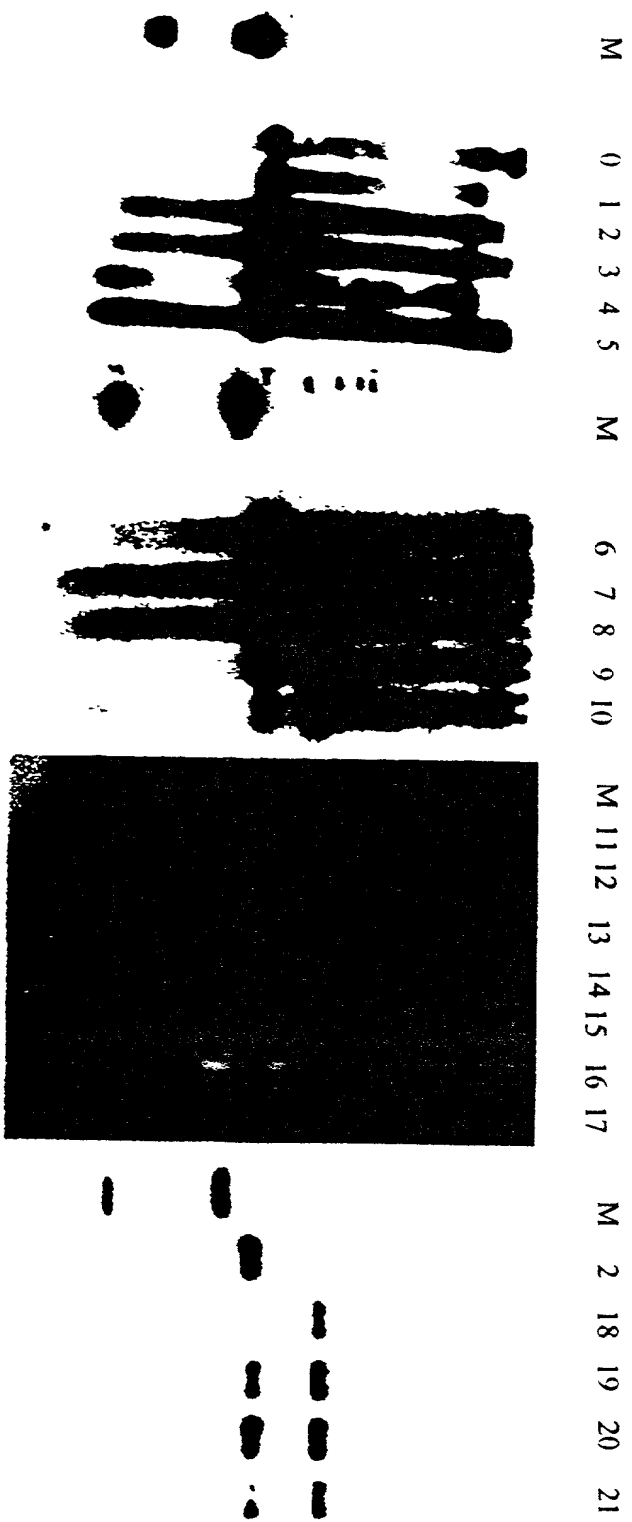
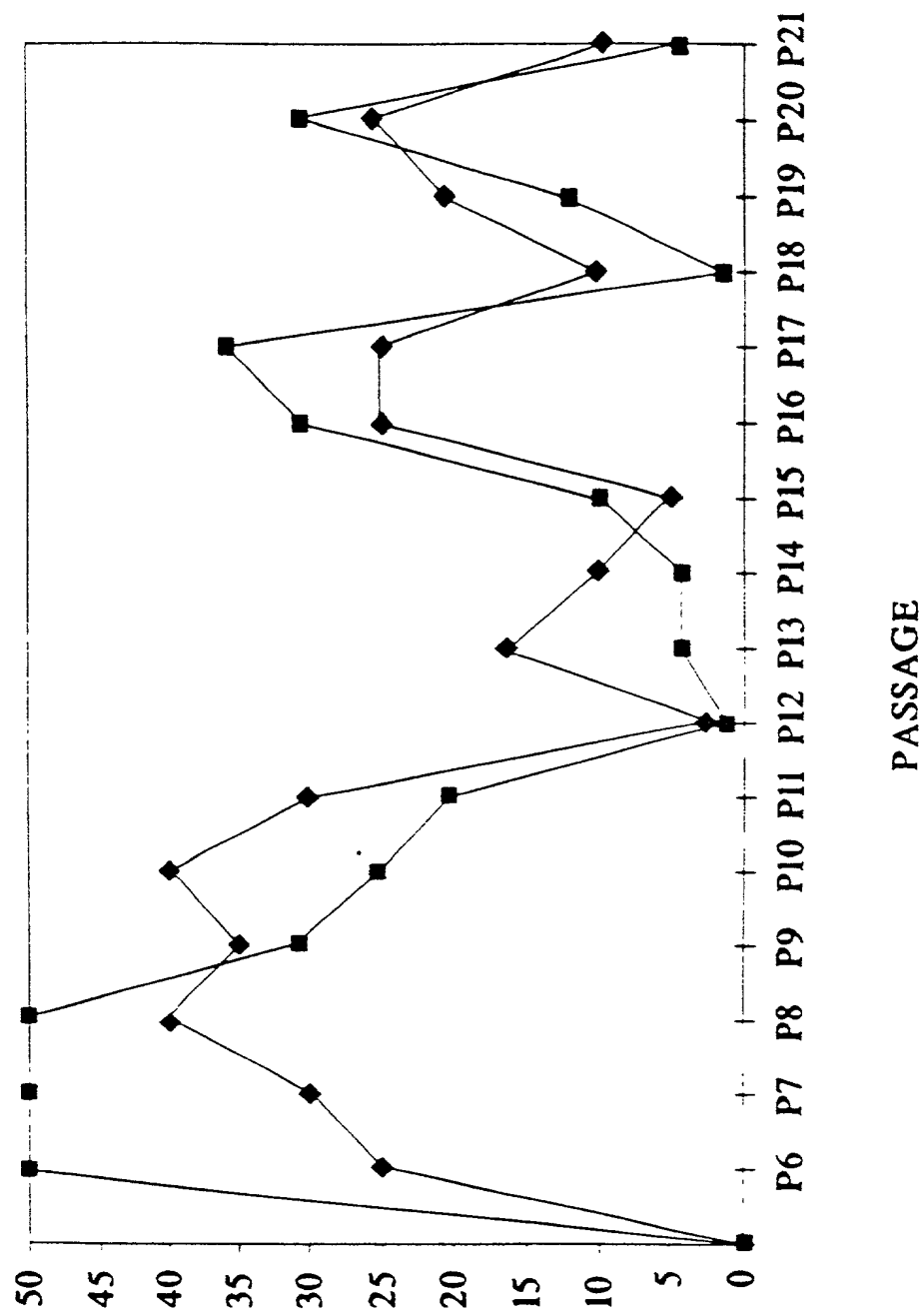


FIGURE 31



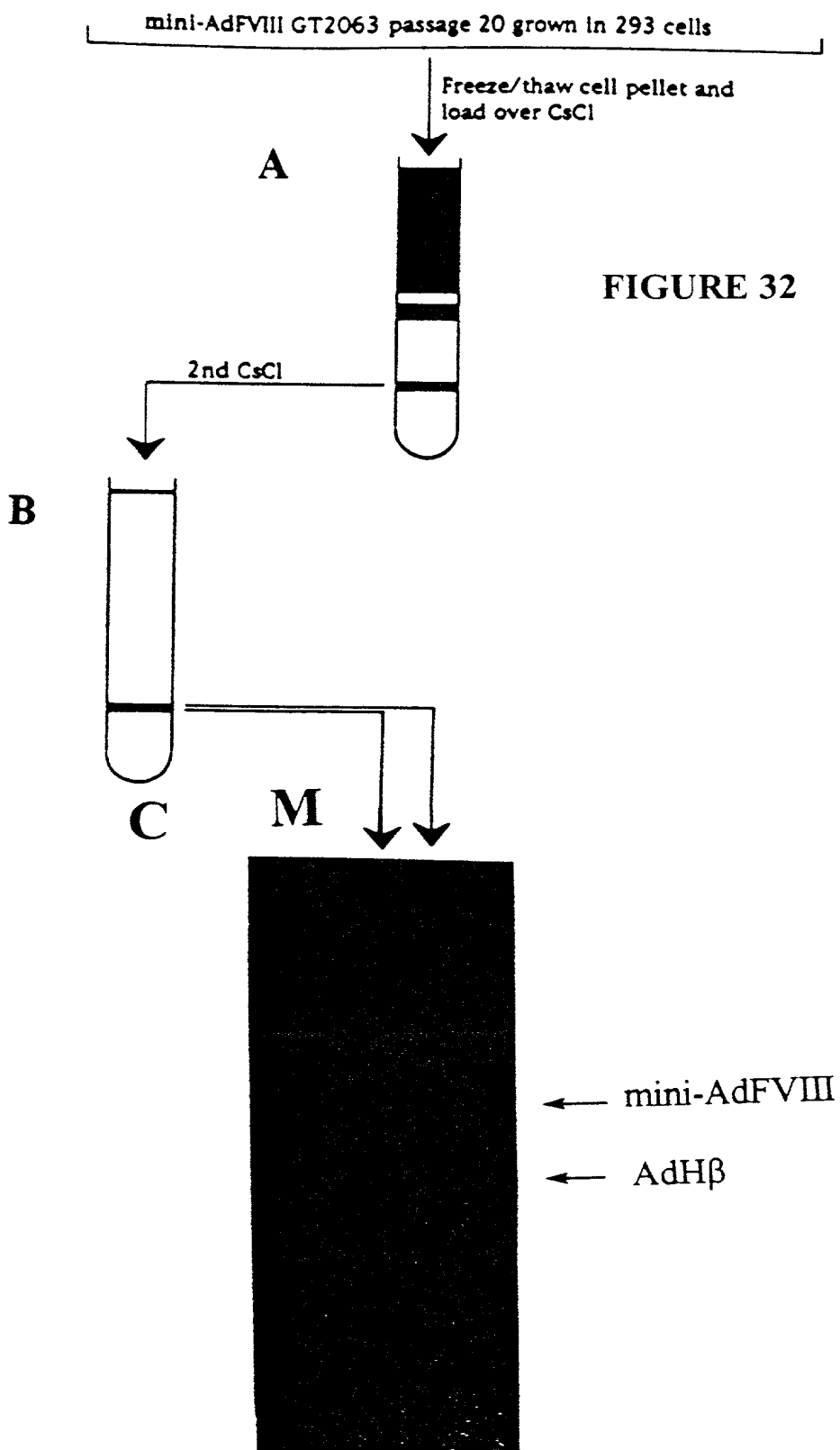


FIGURE 33
MiniAdFVIII-Mediated Expression of hFVIII in Mice

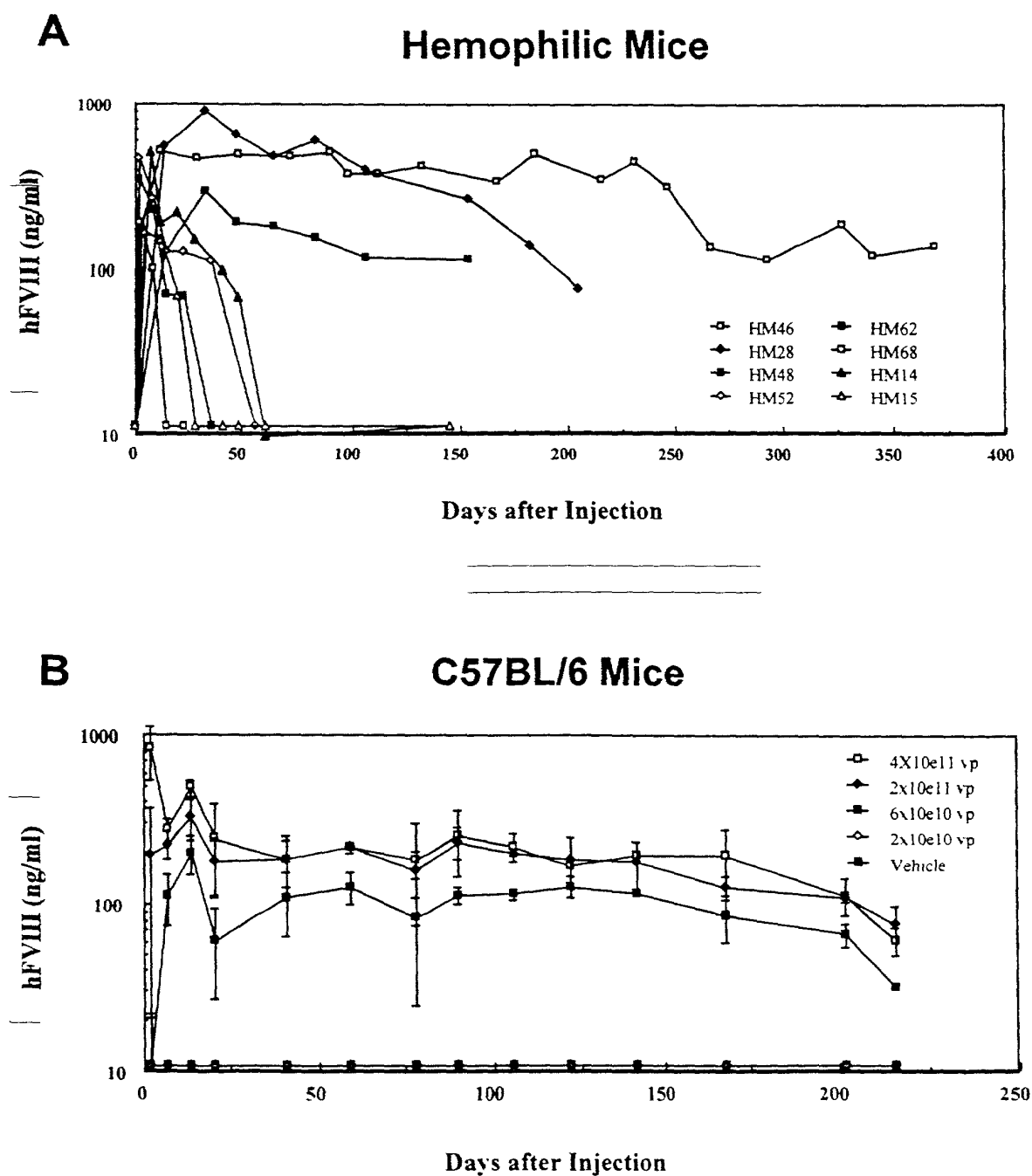


Fig. 8

FIGURE 34

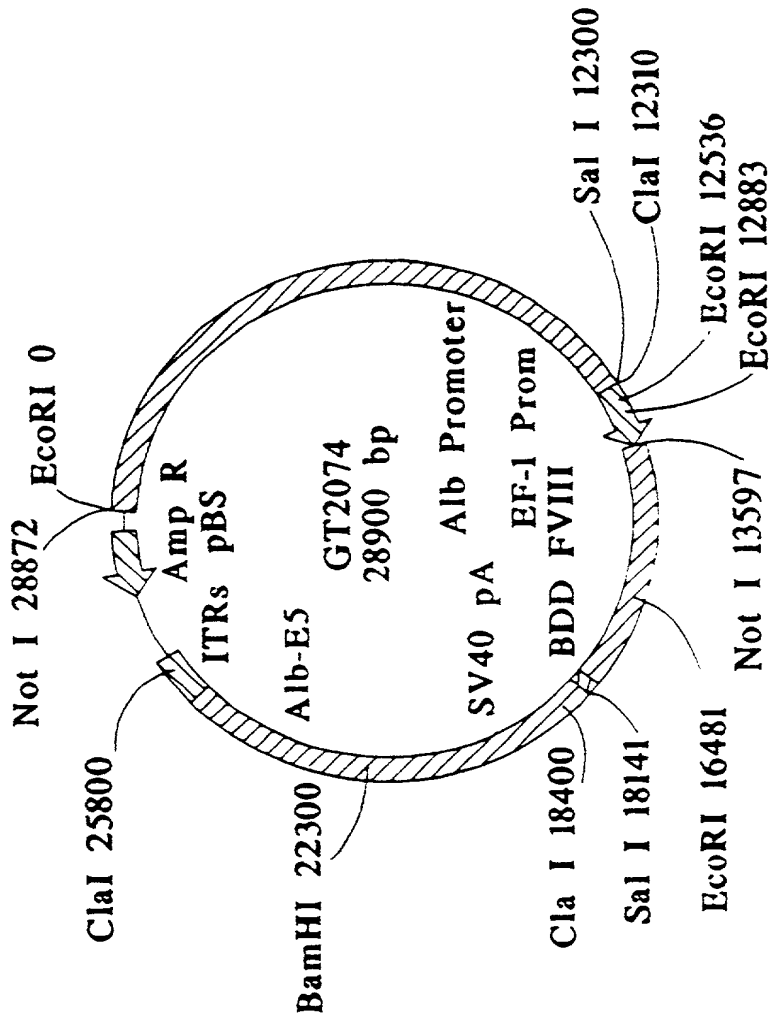


FIGURE 35

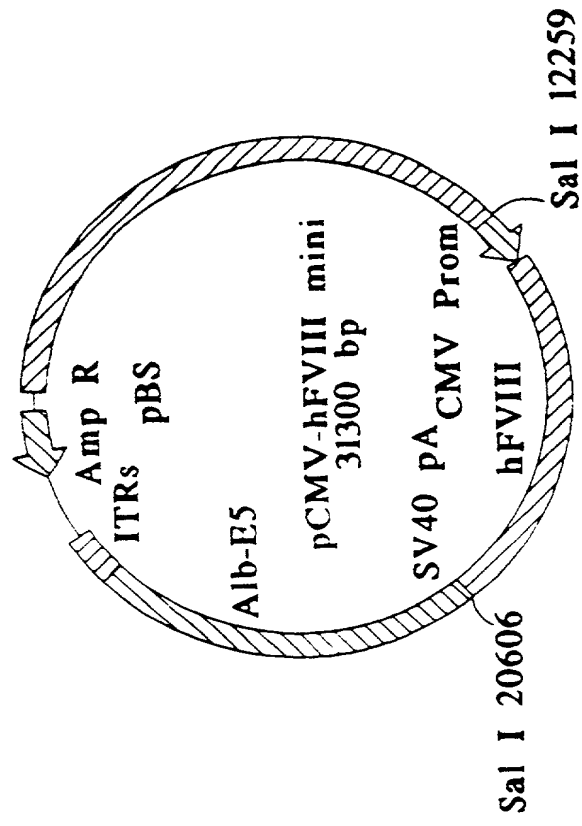


FIGURE 36A

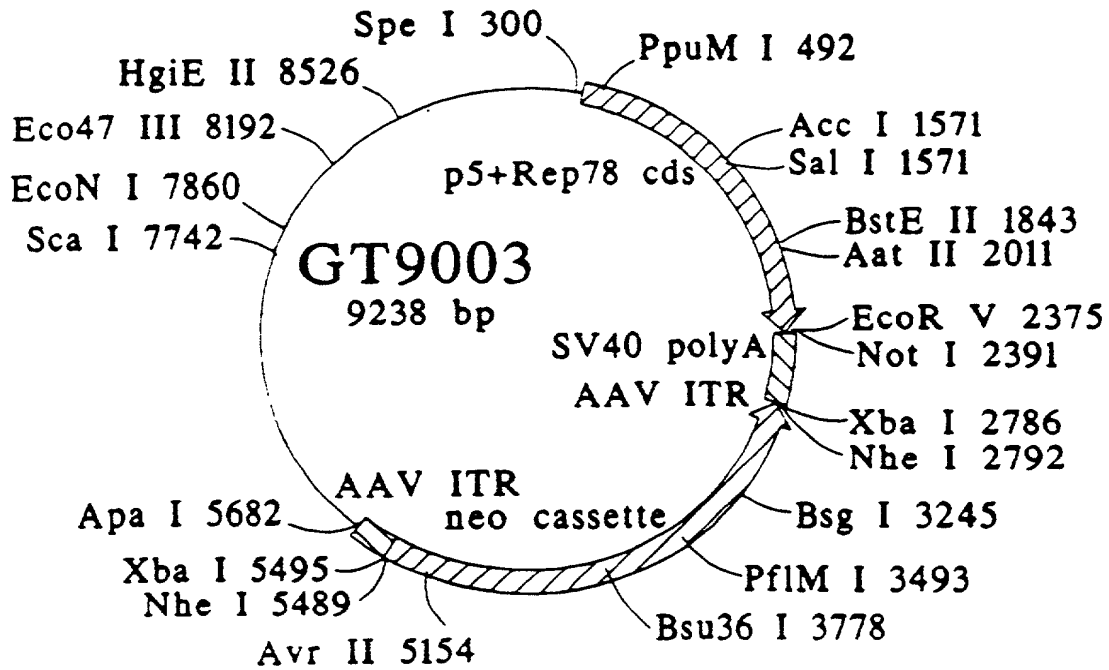


FIGURE 36B

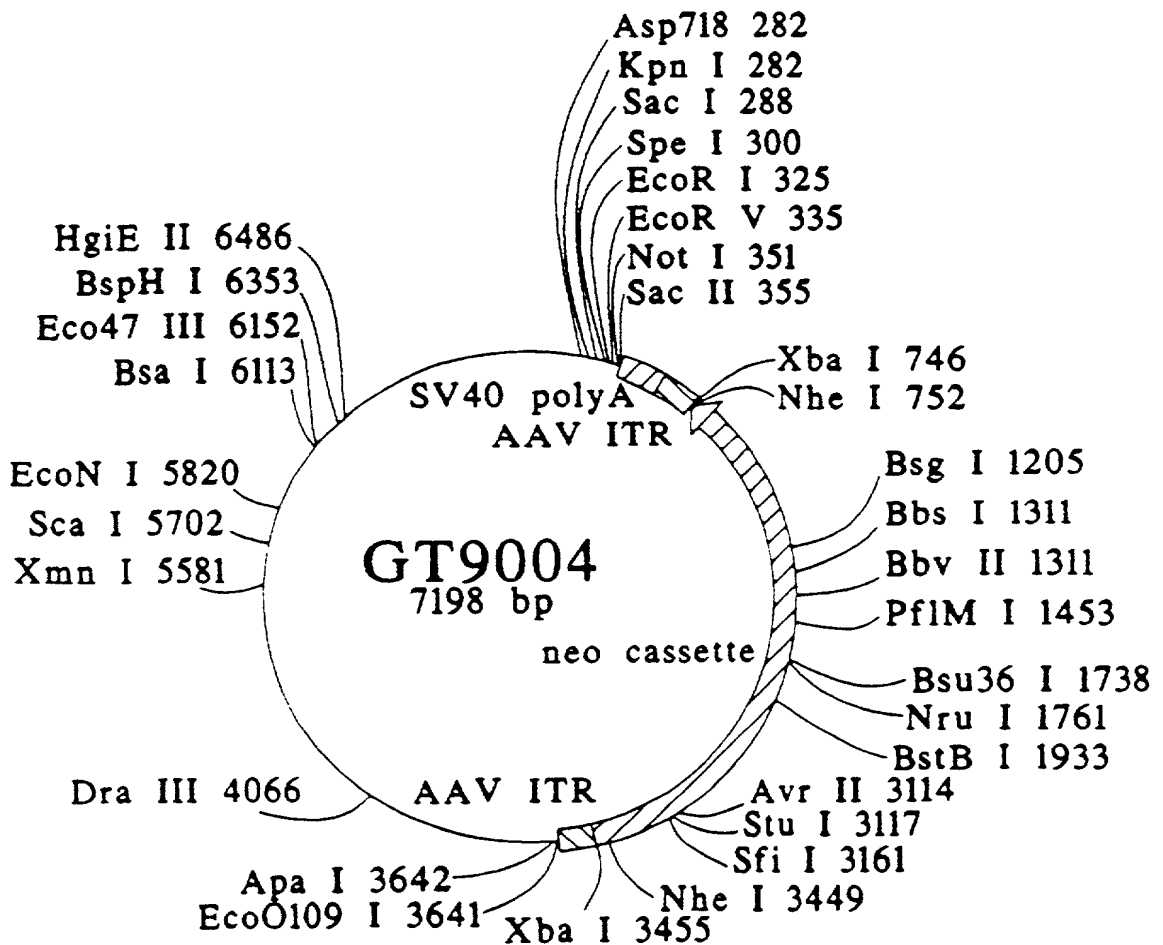


FIGURE 36C

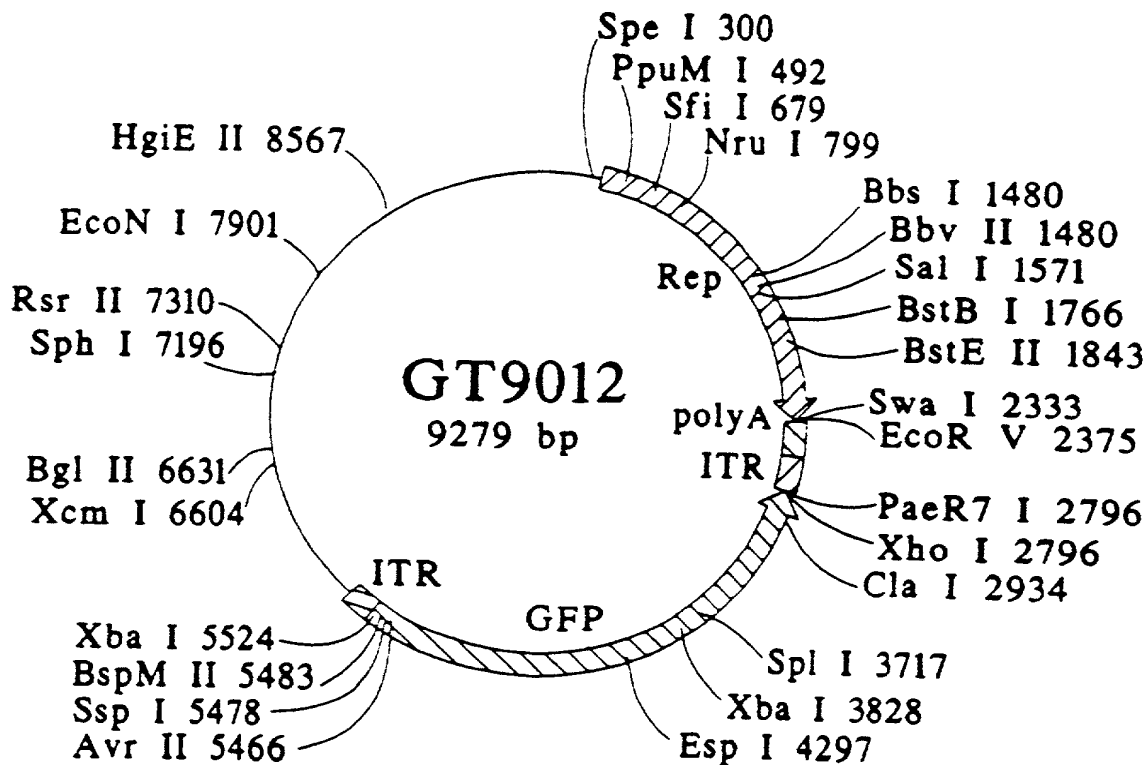


FIGURE 36D

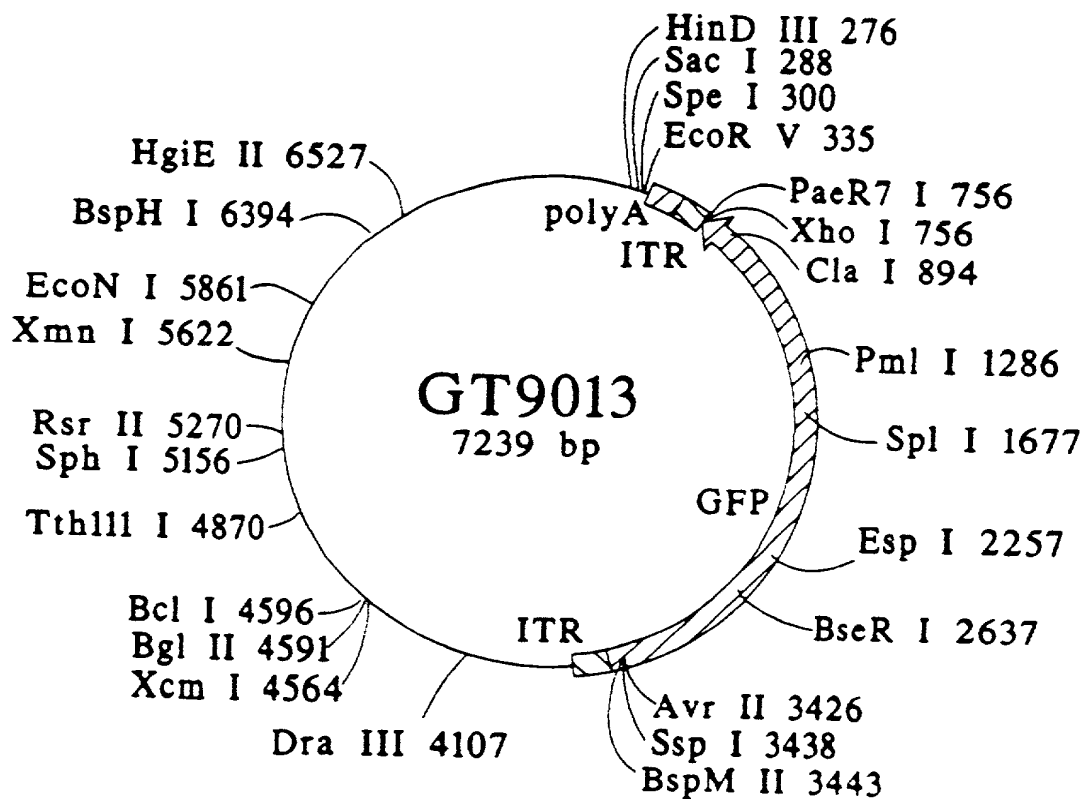


FIGURE 37

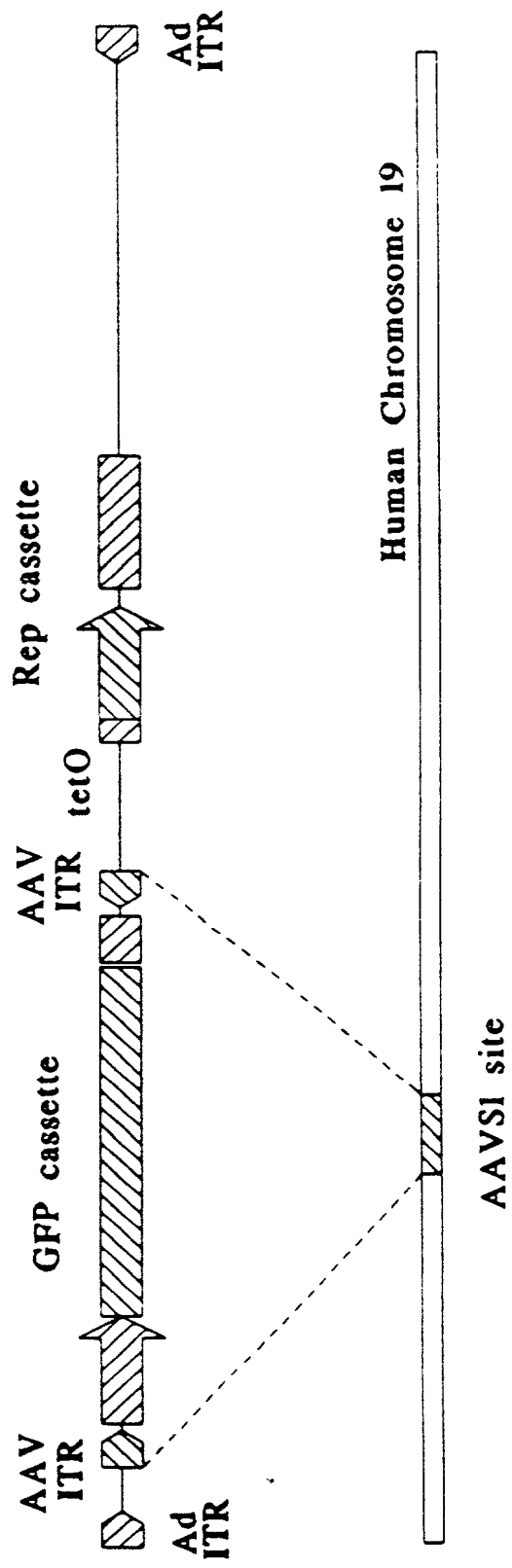


FIGURE 38

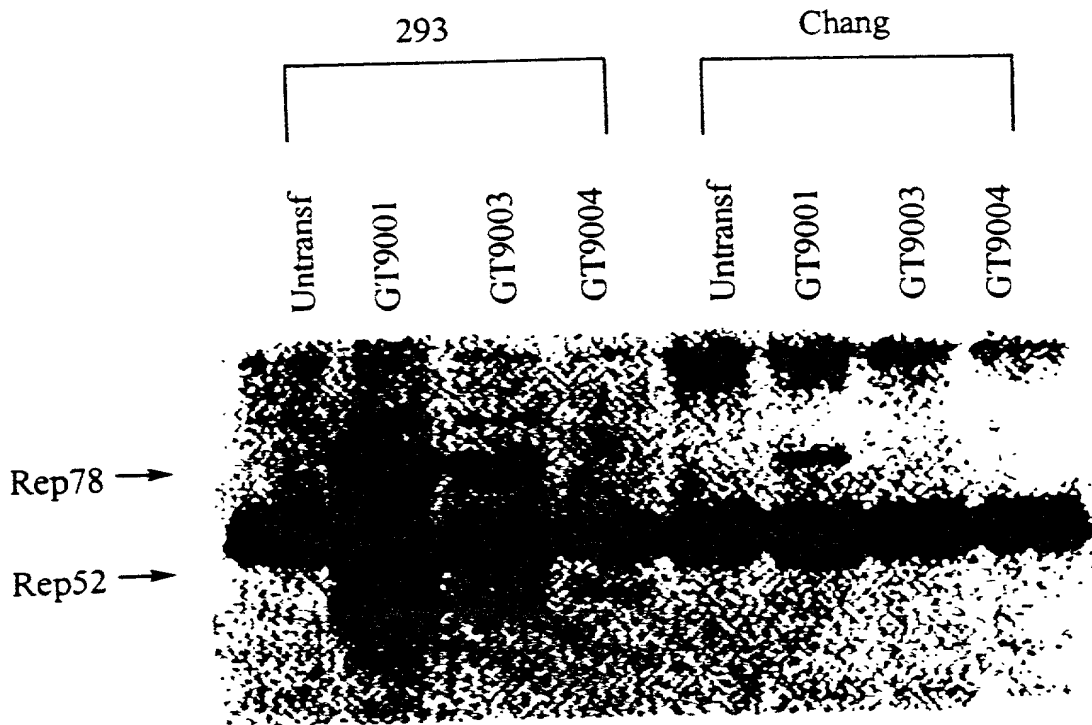
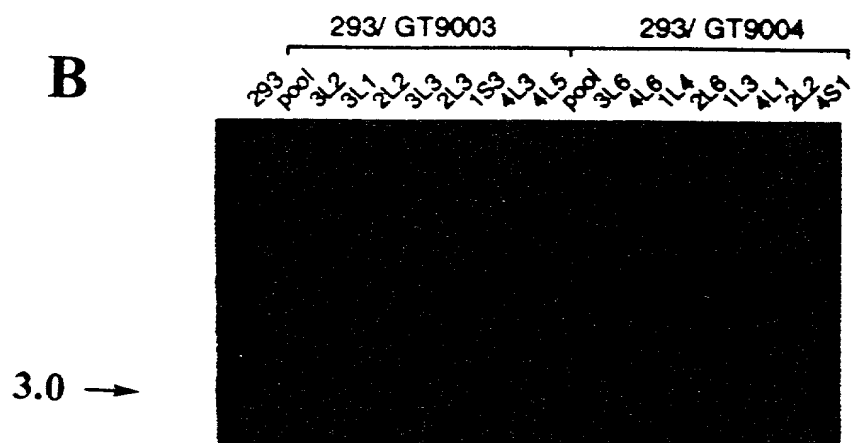
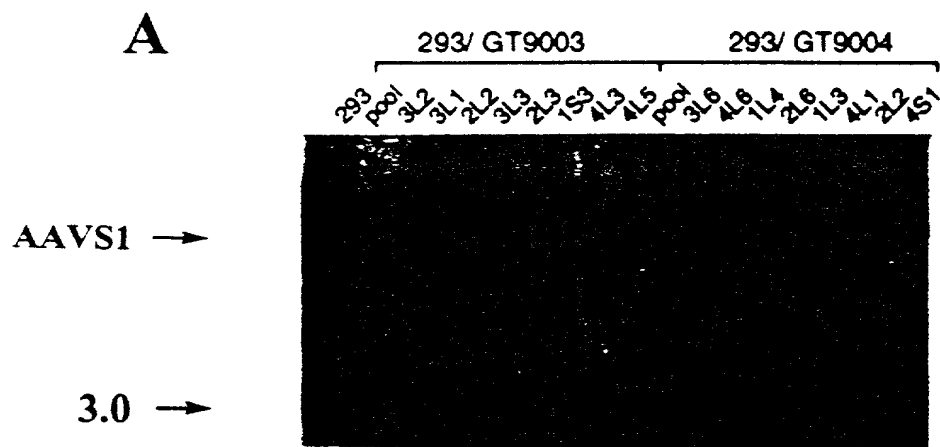


FIGURE 39



	0	1	2	3	4	5	6	7	8	9	10	11	12	13	14	15	16	17	18	19	20	21	22	23	24	25	26	27	28	29	30	31	32	33	34	35	36	37	38	39	40	41	42	43	44	45	46	47	48	49	50	51	52	53	54	55	56	57	58	59	60	61	62	63	64	65	66	67	68	69	70	71	72	73	74	75	76	77	78	79	80	81	82	83	84	85	86	87	88	89	90	91	92	93	94	95	96	97	98	99	100
0	0	1	2	3	4	5	6	7	8	9	10	11	12	13	14	15	16	17	18	19	20	21	22	23	24	25	26	27	28	29	30	31	32	33	34	35	36	37	38	39	40	41	42	43	44	45	46	47	48	49	50	51	52	53	54	55	56	57	58	59	60	61	62	63	64	65	66	67	68	69	70	71	72	73	74	75	76	77	78	79	80	81	82	83	84	85	86	87	88	89	90	91	92	93	94	95	96	97	98	99	100

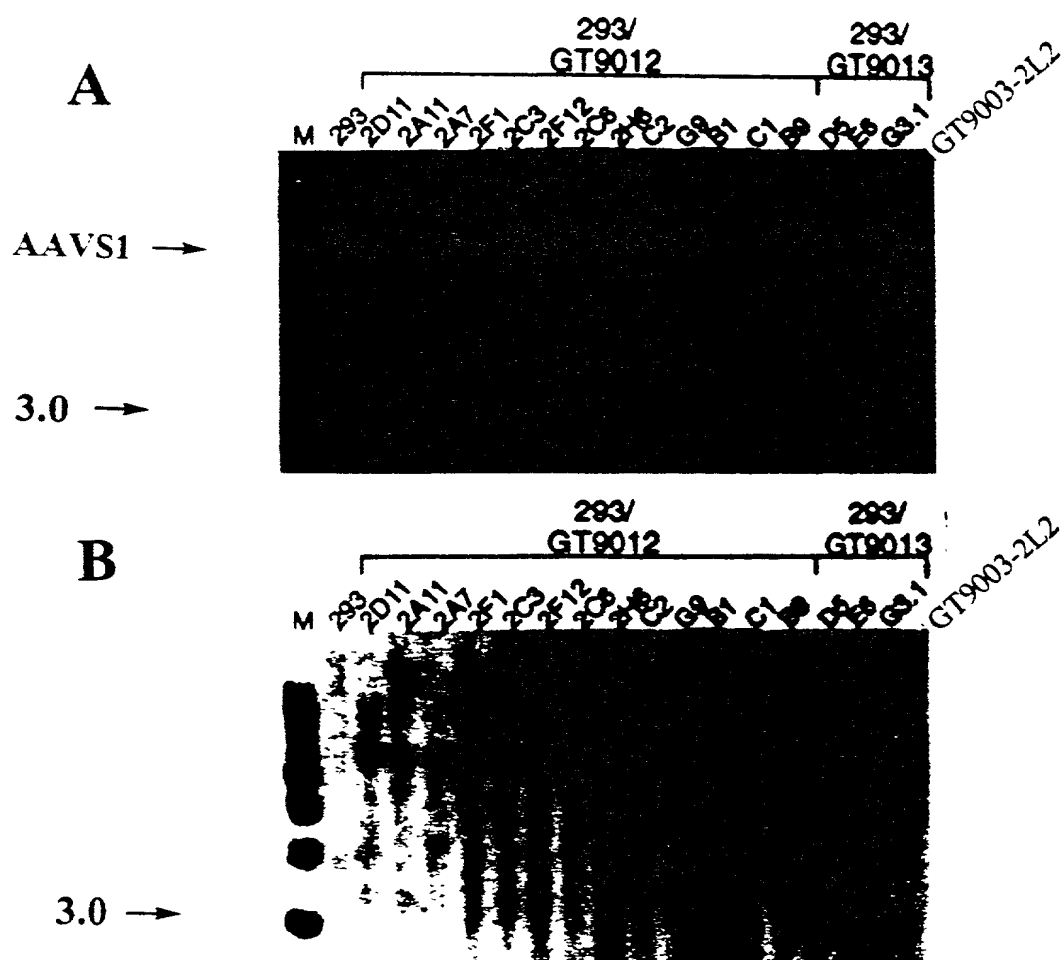


FIGURE 41



FIGURE 42

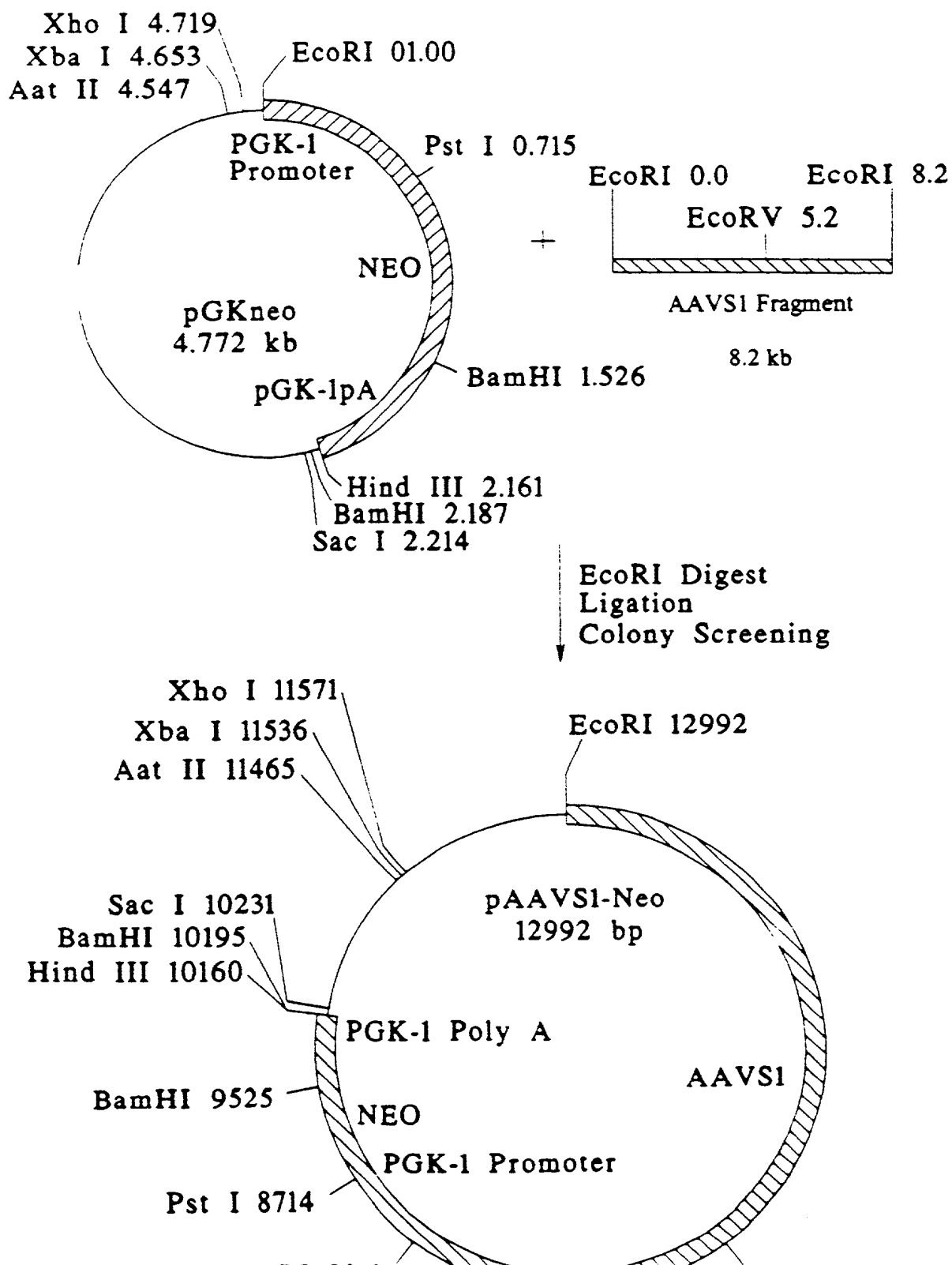


FIGURE 43

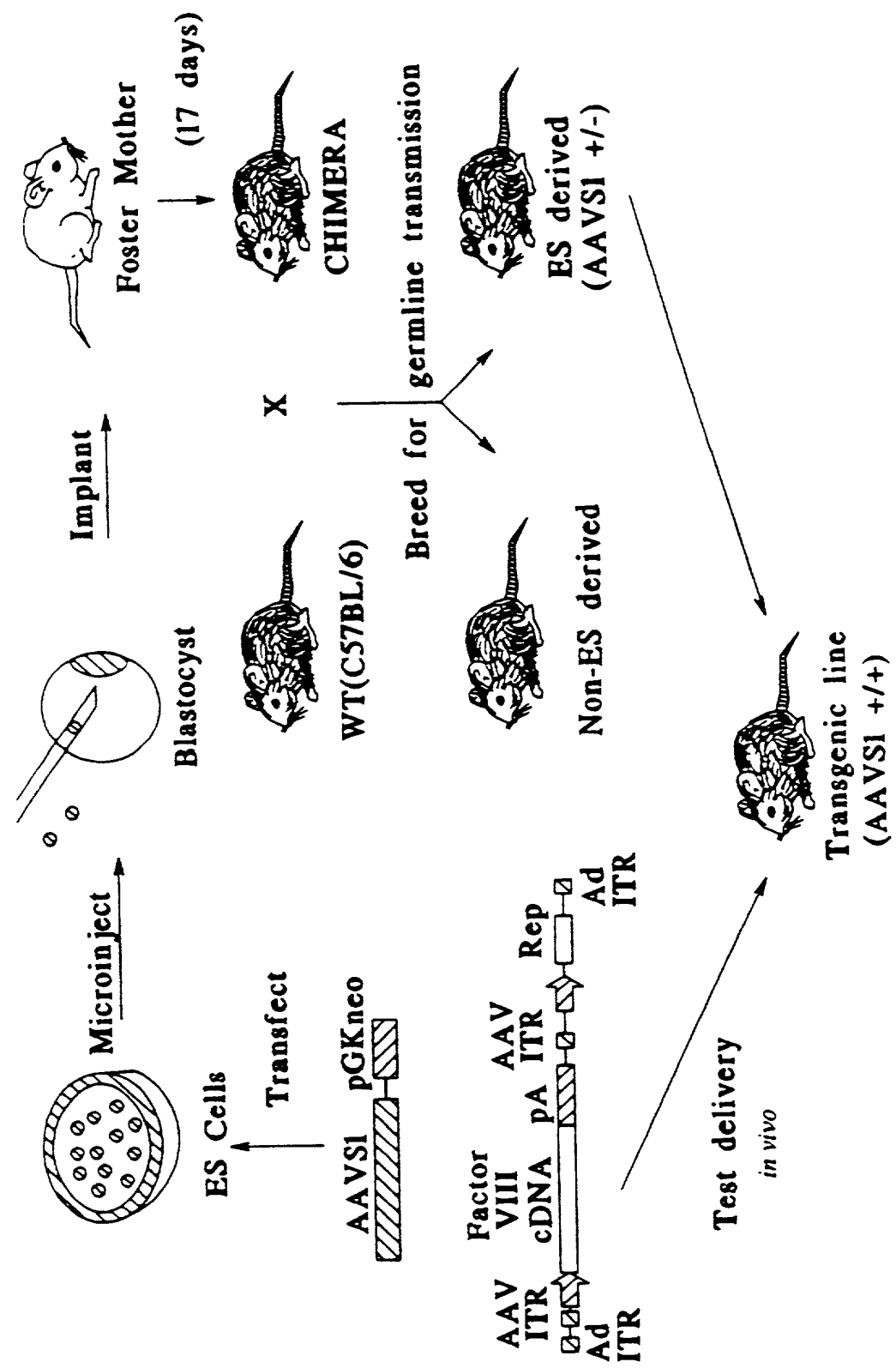


FIGURE 44

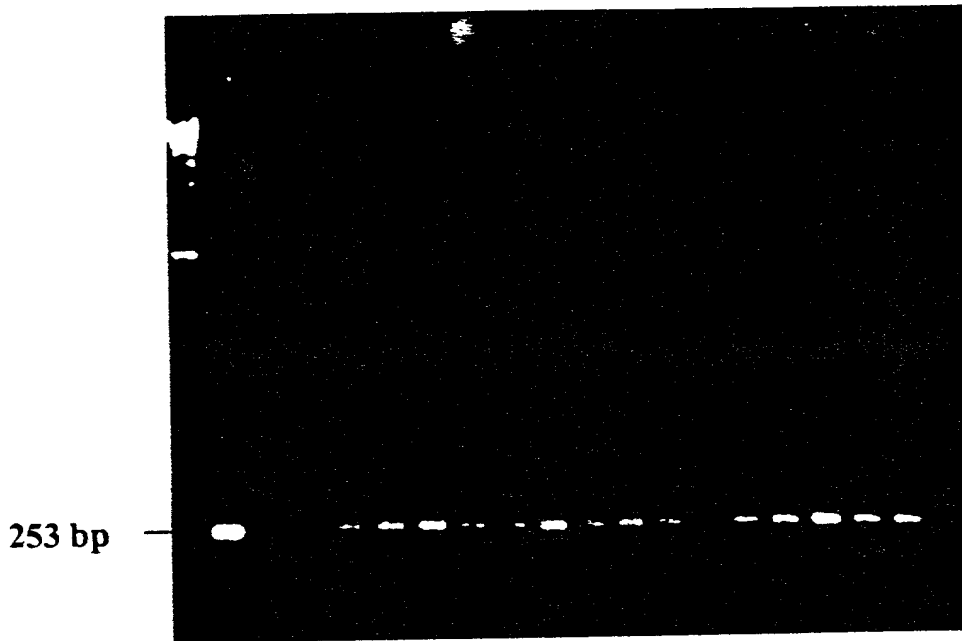


FIGURE 45

4 3.16 B7

5.2 kb →

3.0 kb →



FIGURE 46

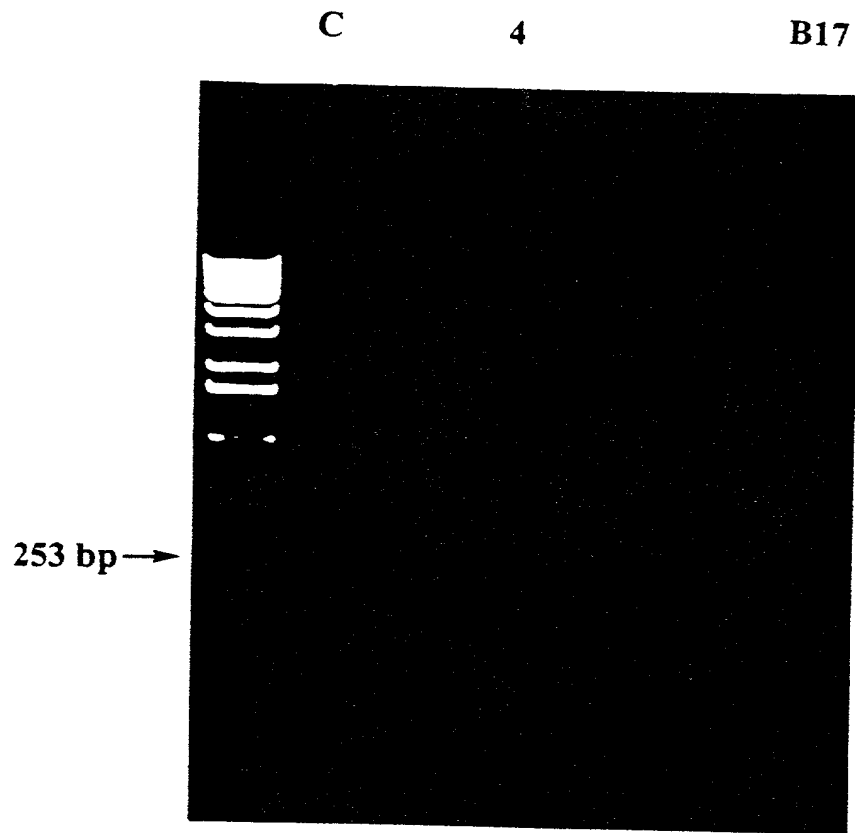


FIGURE 47

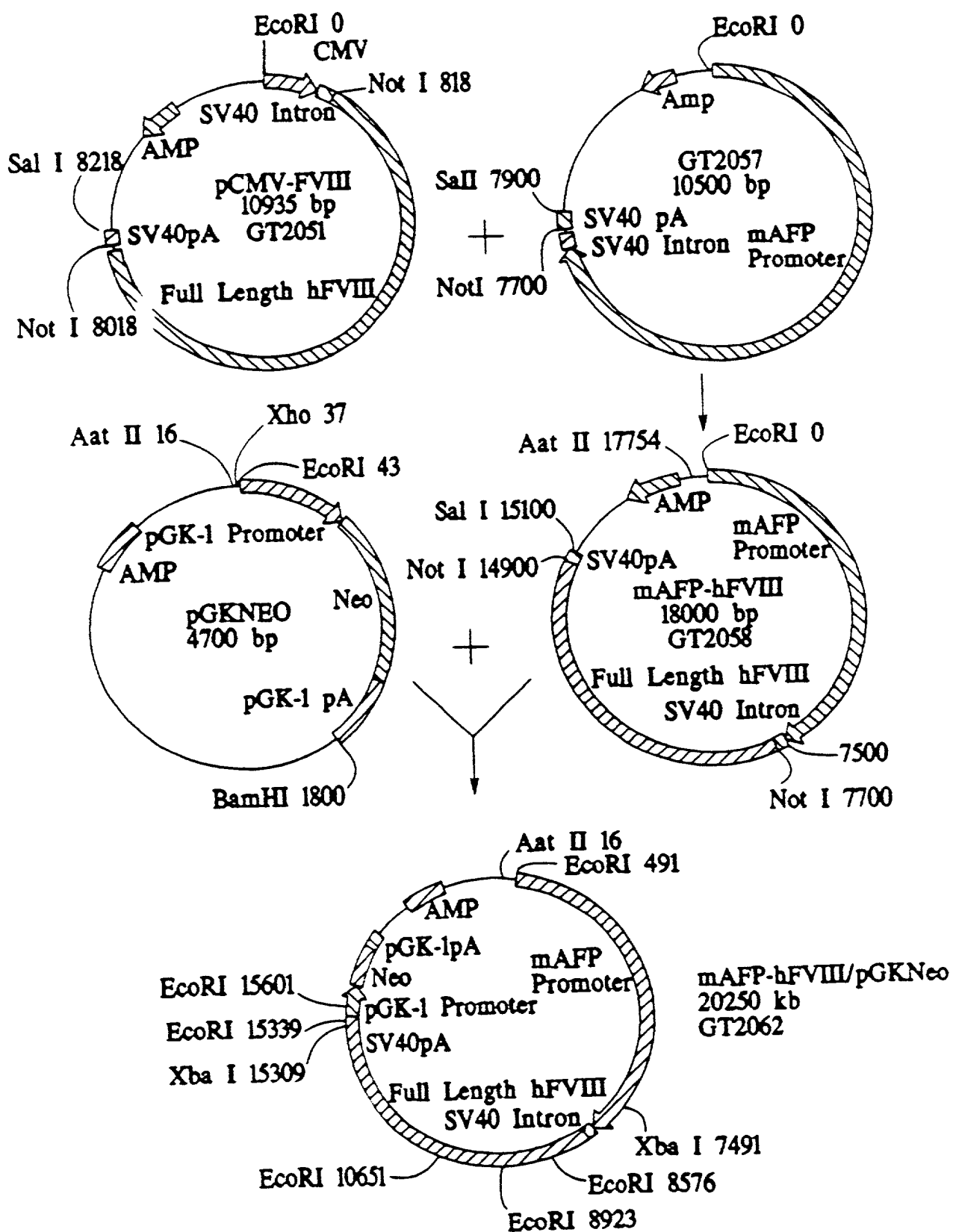


FIGURE 48

ES 19 22 23 33

7.8 kb -



FIGURE 49

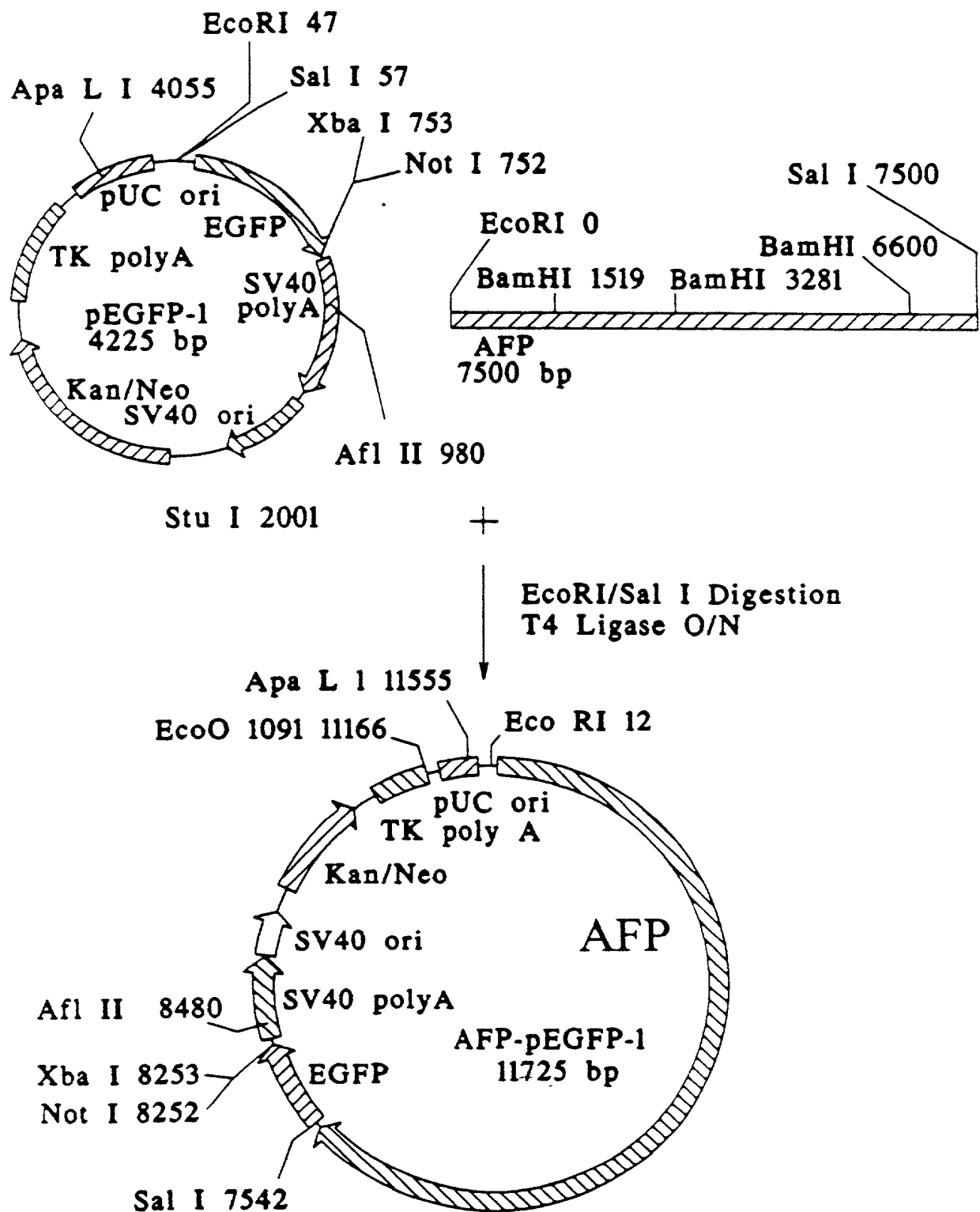


FIGURE 50

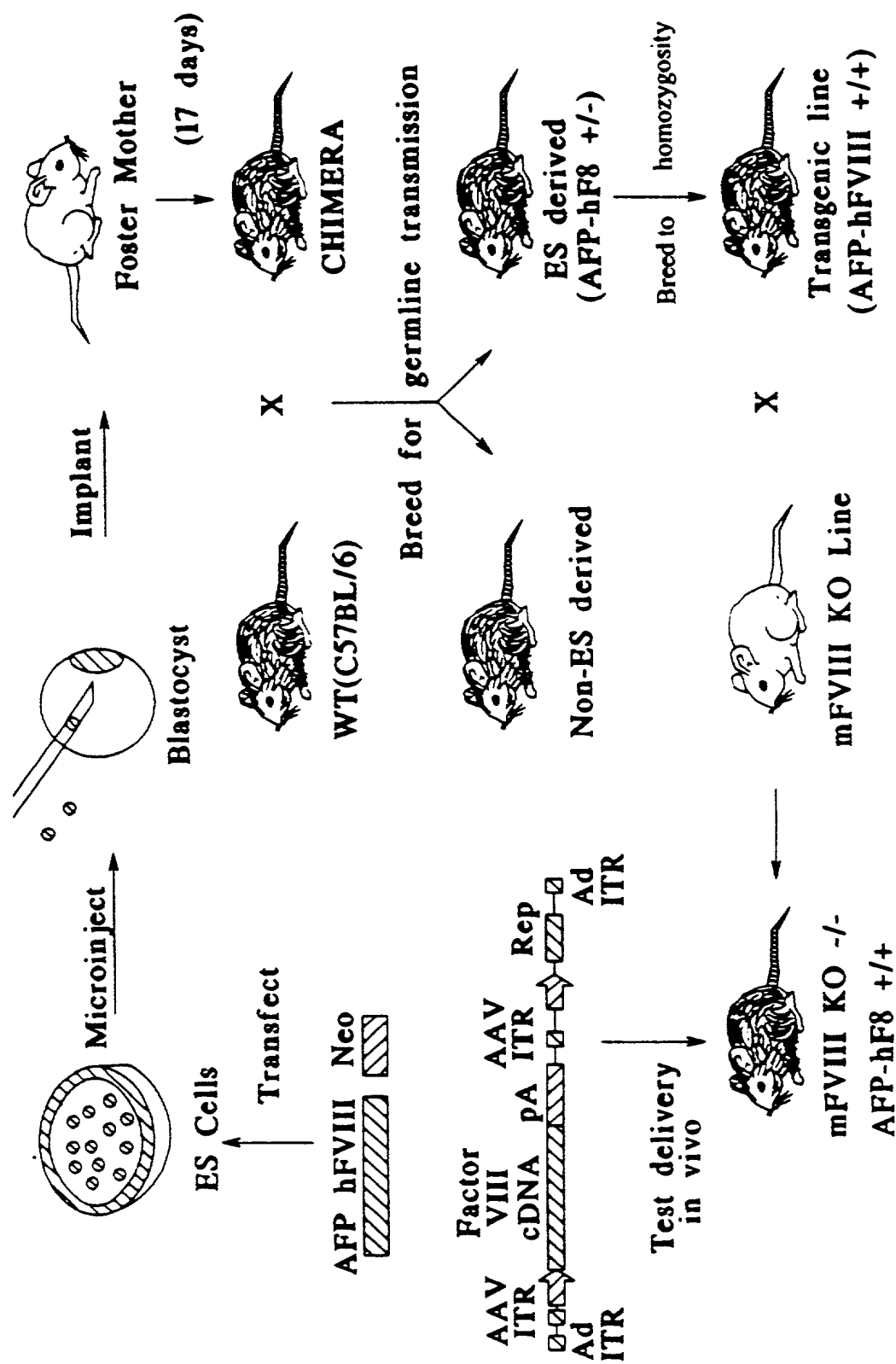


FIGURE 51

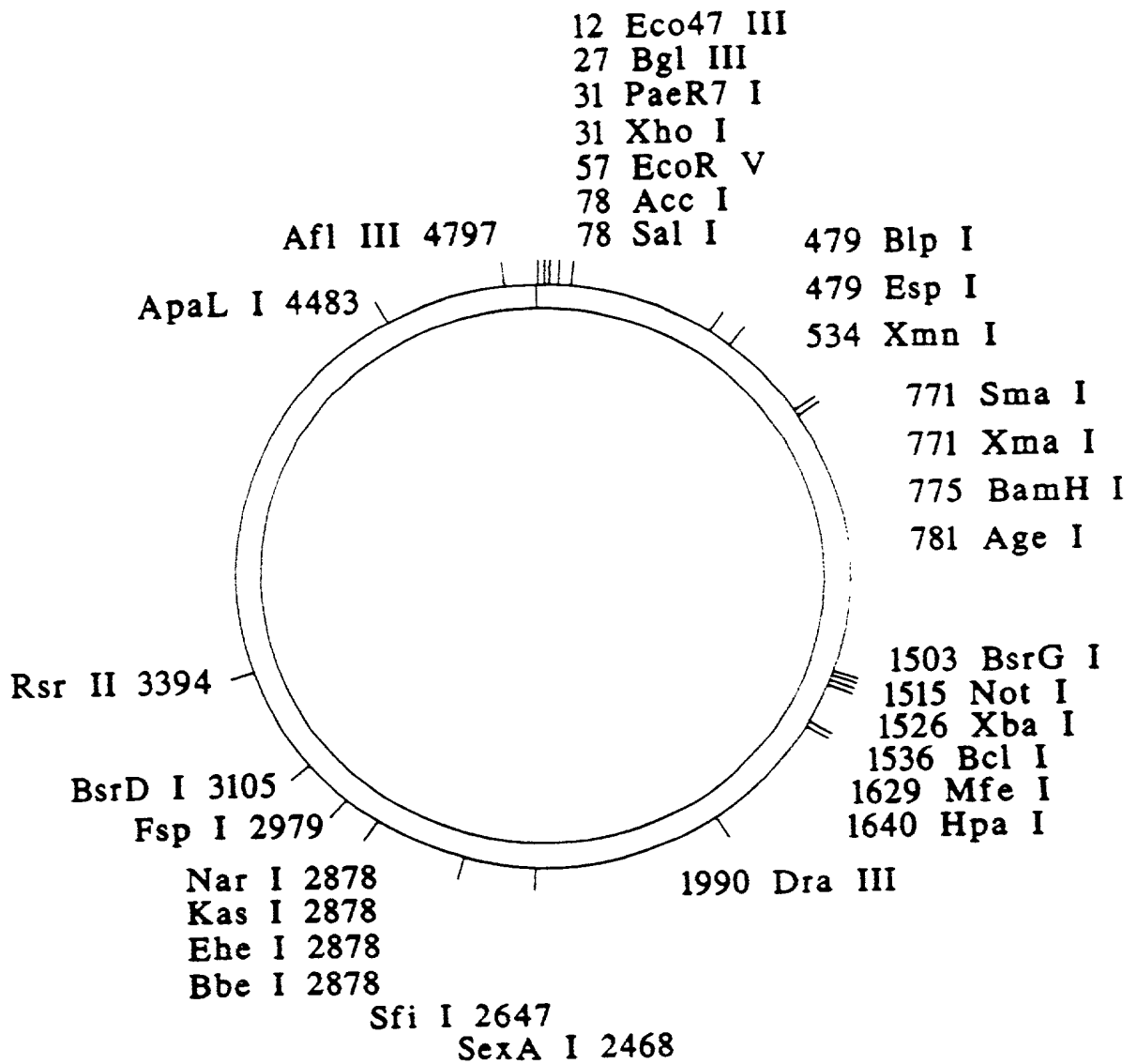


FIGURE 52

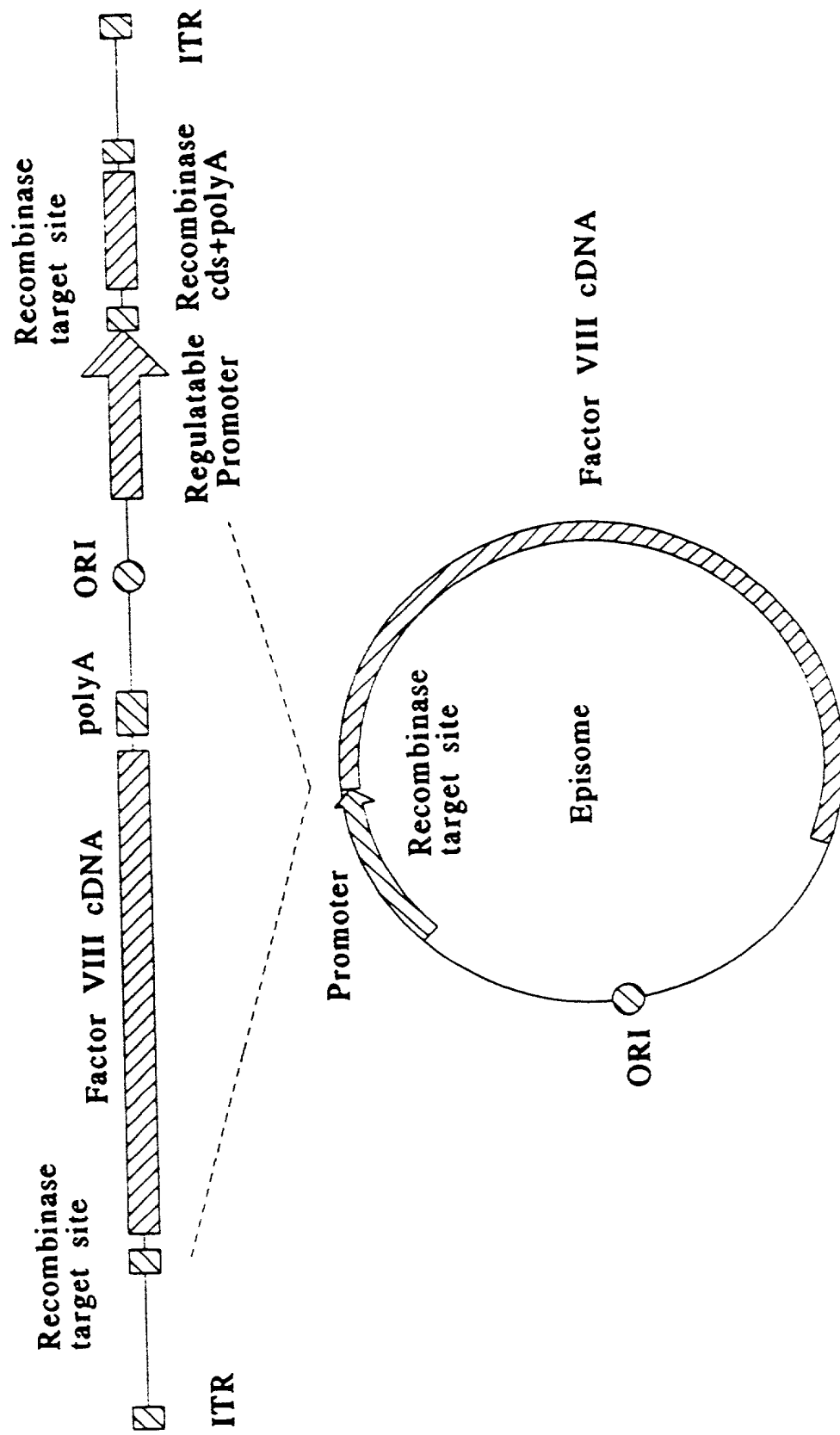


FIGURE 53

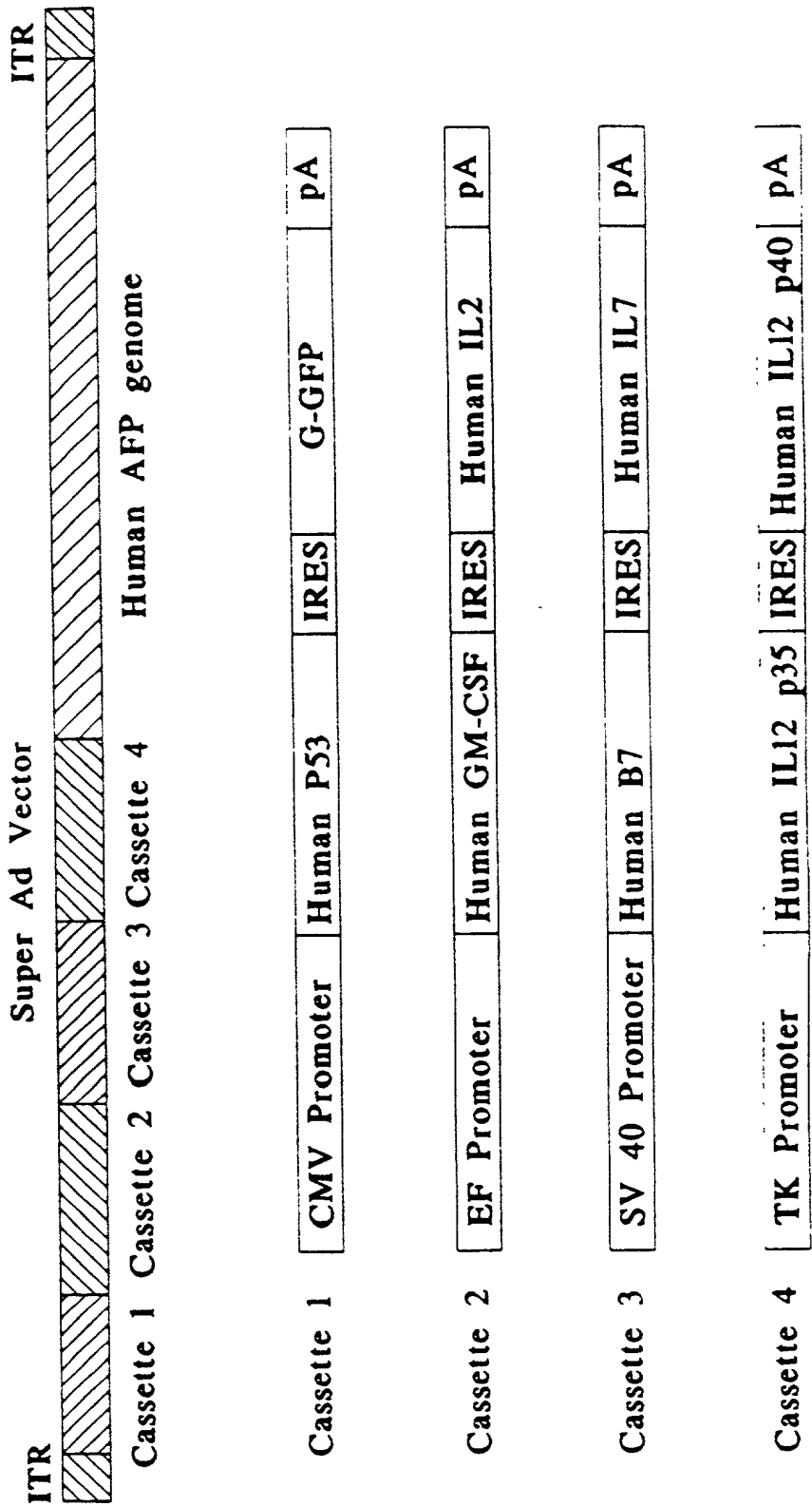


FIGURE 54

